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Clinical Trials Appendices

R&D Pipeline – New Molecular Entities(*)

Phase 1 (Total:17)		Phase 2 (Total:7)		Phase 3 (Total:7)	Registration (Total:2)
SAR441344 Anti-CD40L mAb Multiple Sclerosis	**BIVV001**(4) rFVIIIFc – vWF – XTEN(5) Hemophilia A	**SAR440340**(**) Anti-IL33 mAb Atopic Dermatitis	**SAR422459**(**)(12) ABCA4 gene therapy Stargardt Disease	**isatuximab** Anti-CD38 mAb 3L Relapsing Refractory MM (ICARIA)	**cemiplimab**(**) PD-1 inhibitor mAb Advanced CSCC (EU)
O **REGN5458**(1) Anti BCMA-CD3 bispecific mAb RRMM	**ST400**(6) ZFN Gene Editing Technology Beta thalassemia	**SAR156597** IL4/IL13 bi-specific mAb Systemic Scleroderma	**HIV** Viral vector prime & rgp120 boost vaccine	**avalglucosidase alfa** Neo GAA Pompe Disease	**Zynquista**TM(**) Oral SGLT-1&2 inhibitor Type 1 Diabetes (U.S./EU)
O **REGN4018**(1) Anti MUC16-CD3 bispecific mAb Ovarian Cancer	**BIVV003**(6) ZFN Gene Editing Technology Sickle Cell Disease	R **olipudase alfa** rhASM Acid Sphingomyelinase Deficiency(10)	**SP0232**(13)(**) Respiratory syncytial virus Monoclonal Antibody	**venglustat** Oral GCS inhibitor ADPKD(14)	
SAR408701 Maytansin-loaded anti-CEACAM5 mAb Solid Tumors	**SAR443060**(7) RIPK1 inh(8) Amyotrophic Lateral Sclerosis	**SAR339375**(11) miRNA-21 Alport Syndrome		**fitusiran** RNAi therapeutic targeting anti-thrombin Hemophilia A and B	
SAR439459 anti-TGFβ mAb Advanced Solid Tumors	**SAR442168**(9)(**) BTK inhibitor Multiple Sclerosis			**sutimlimab**(15) Anti Complement C1s mAb Cold Agglutinin Disease	
SAR439859 SERD Metastatic Breast Cancer	**Herpes Simplex Virus Type 2** HSV-2 vaccine			**SAR341402** Rapid acting insulin Type 1/2 Diabetes	
SAR442720(2) SHP2 inhibitor Solid Tumors	**Respiratory syncytial virus** Infants Vaccines			**efpeglenatide**(**) Long-acting GLP-1 agonist Type 2 Diabetes	
SAR440234 T cell engaging multi spe mAb Leukemia	**Next Gen PCV** Pneumococcal Conjugate Vaccines				
SAR441000(3) Cytokine mRNA Melanoma					

R Registrational Study (other than Phase 3)

O Opt-in rights products for which rights have not been exercised yet

- Immuno-inflammation
- Oncology
- Rare Diseases
- Rare Blood Disorders
- MS & Neuro
- Diabetes
- Cardiovascular & metabolism
- Vaccines

(1) Regeneron product for which Sanofi has opt-in rights
(2) Developed in collaboration with REVOLUTION Medicines; also known as RMC-4630
(3) Developed in collaboration with BioNtech
(4) Sanofi Product for which Sobi has opt-in rights
(5) Recombinant Coagulation Factor VIII Fc – von Willebrand Factor – XTEN Fusion protein
(6) Developed in collaboration with Sangamo
(7) Also known as DNL747
(8) Receptor-interacting serine/threonine-protein kinase 1
(9) Also known as PRN2246
(10) Also known as Niemann Pick type B
(11) Regulus product for which Sanofi has decided to opt-in
(12) Identification of out-licensing partner ongoing
(13) Also known as MEDI8897
(14) Autosomal Dominant Polycystic Kidney Disease
(15) Also Known as BIVV009
(*) Phase of projects determined by clinicaltrials.gov disclosure timing
(**) Partnered and/or in collaboration – Sanofi may have limited or shared rights on some of these products

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Additional Indications(*)

Phase 1 (Total:5)	Phase 2 (Total:17)		Phase 3 (Total:23)		Registration (Total:3)
[O] **cemiplimab(**) + REGN4018(1)** PD-1 inhibitor mAb + Anti-MUC16-CD3 bispecific mAb - Ovarian Cancer	**dupilumab(**)** Anti-IL4Rα mAb Grass Immunotherapy	**isatuximab + cemiplimab(**)** Anti-CD38 mAb + PD-1 inhibitor mAb Advanced Malignancies	**dupilumab(**)** Anti-IL4Rα mAb Asthma 6 - 11 years old	**isatuximab** Anti-CD38 mAb Newly Diagnosed MM Te(5) (GMMG)	**dupilumab(**)** Anti-IL4Rα mAb Asthma 12y+ (EU)
SAR439859 SERD + Palbociclib Metastatic Breast Cancer	[R] **sarilumab(**)** Anti-IL6R mAb Polyarticular Juvenile Idiopathic Arthritis	**isatuximab + cemiplimab(**)** Anti-CD38 mAb + PD-1 inhibitor mAb Lymphoma	**dupilumab(**)** Anti-IL4Rα mAb Nasal Polyposis	**isatuximab** Anti-CD38 mAb 1L Newly Diagnosed MM Ti(6) (IMROZ)	**Dupixent®(**)** dupilumab Atopic Dermatitis 12 – 17 years old (U.S./EU)
SAR439459 + cemiplimab(**) Anti-TGFβ mAb + PD-1 inhibitor mAb Advanced Solid Tumors	**sarilumab(**)** Anti-IL6R mAb Systemic Juvenile Arthritis	**isatuximab + atezolizumab(**)** Anti-CD38 mAb + PD-L1 inhibitor mAb Advanced Malignancies	**Dupixent®(**)** dupilumab Atopic Dermatitis 6 – 11 years old	**Cerdelga®** eliglustat Gaucher Type 1, switch from ERT - Pediatric	**Praluent®(**)** alirocumab CV events reduction (U.S./EU)
sutimlimab(2) Anti Complement C1s mAb Idiopathic Thrombocytopenic Purpura	**SAR440340(**)** Anti-IL33 mAb COPD	**isatuximab + atezolizumab(**)** Anti-CD38 mAb + PD-L1 inhibitor mAb Solid Tumors	**Dupixent®(**)** dupilumab Atopic Dermatitis 6 months - 5 years old	**Aubagio®** teriflunomide Relapsing Multiple Sclerosis - Pediatric	
SAR443060(3) RIPK1 inh(4) Alzheimer's Disease	**SAR440340(**)** Anti-IL33 mAb Asthma	**venglustat** Oral GCS inhibitor Fabry Disease	**dupilumab(**)** Anti-IL4Rα mAb Eosinophilic Esophagitis	**Lemtrada®** alemtuzumab Relapsing Remitting Multiple Sclerosis - Pediatric	
	dupilumab (**)+ AR101 Anti-IL4Rα mAb Peanut Allergy - Pediatric	**venglustat** Oral GCS inhibitor Gaucher Type 3	**sarilumab(**)** Anti-IL6R mAb Giant Cell Arteritis	**Zynquista™(**)** Oral SGLT-1&2 inhibitor Type 2 Diabetes	
	[R] **cemiplimab(**)** PD-1 inhibitor mAb Advanced Basal Cell Carcinoma	**venglustat** Oral GCS inhibitor Gaucher related Parkinson's Disease	**sarilumab(**)** Anti-IL6R mAb Polymyalgia Rheumatica	**Zynquista™(**)** Oral SGLT-1&2 inhibitor Worsening Heart Failure in Diabetes	
	isatuximab + cemiplimab(**) Anti-CD38 mAb + PD-1 inhibitor mAb Relapsing Refractory MM	**Rabies VRVg** Purified vero rabies vaccine	**cemiplimab(**)** PD-1 inhibitor mAb 1L NSCLC	**Praluent®(**)** alirocumab LDL-C reduction - Pediatric	
		SP0173 Tdap booster US Tdap booster	**cemiplimab(**) + chemotherapy** PD-1 inhibitor mAb 1L NSCLC	**Fluzone® QIV HD** Quadrivalent inactivated Influenza vaccine - High dose	
			cemiplimab(**) PD-1 inhibitor mAb 2L Cervical Cancer	**Men Quad TT** Advanced generation meningococcal ACYW conjugate vaccine	
			isatuximab Anti-CD38 mAb 1-3L Relapsing Refractory MM (IKEMA)	**Pediatric pentavalent vaccine** DTP-Polio-Hib Japan	
				Shan 6 DTP-HepB-Polio-Hib Pediatric hexavalent vaccine	

- Immuno-inflammation
- Oncology
- Rare Diseases
- Rare Blood Disorders
- MS & Neuro
- Diabetes
- Cardiovascular & metabolism
- Vaccines

[R] Registrational study (other than Phase 3)

[O] Opt-in rights products for which rights have not been exercised yet

(1) Regeneron product for which Sanofi has opt-in rights
(2) Also known as BIVV009
(3) Also known as DNL747
(4) Receptor-interacting serine/threonine-protein kinase 1
(5) Transplant eligible
(6) Transplant ineligible
(*) Phase of projects determined by clinicaltrials.gov disclosure timing
(**) Partnered and/or in collaboration - Sanofi may have limited or shared rights on some of these products

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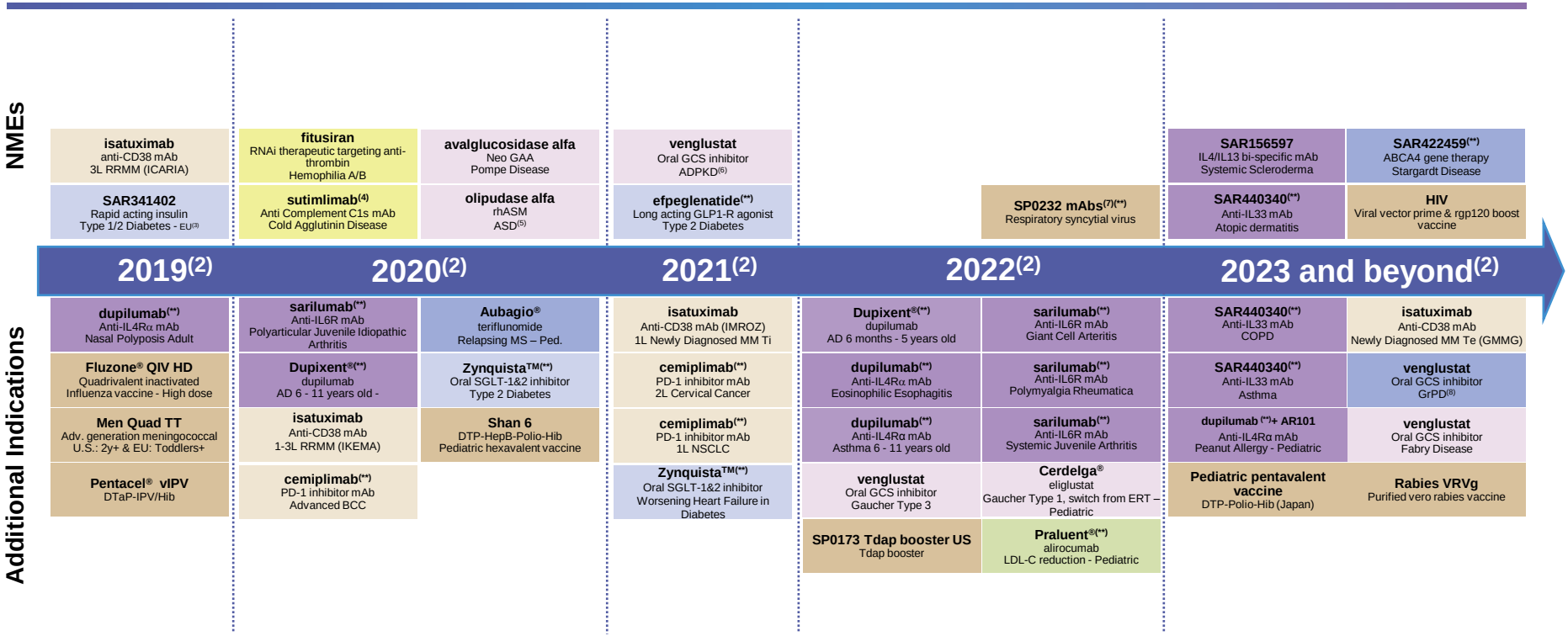
Expected Submission Timeline(1)



(1) Excluding Phase 1
(2) Projects within a specified year are not arranged by submission timing
(3) Submission strategy for the U.S. under evaluation
(4) Also known as BIVV009
(5) Acid Sphingomyelinase Deficiency
(6) Autosomal Dominant Polycystic Kidney Disease
(7) Also known as MEDI8897
(8) Gaucher Related Parkinson's Disease
(**) Partnered and/or in collaboration – Sanofi may have limited or shared rights on some of these products

Immuno-inflammation
Oncology
Rare Diseases
Rare Blood Disorders
MS & Neuro
Diabetes
Cardiovascular & metabolism
Vaccines

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Pipeline Movements Since Q3 2018

Additions

Registration

Phase 3

Shan 6
DTP-HepB-Polio-Hib
Pediatric hexavalent vaccine

Phase 2

SAR440340(**)
Anti-IL33 mAb
Atopic Dermatitis

isatuximab + cemiplimab(**)
Anti-CD38 mAb + PD-1 inhibitor mAb
Lymphoma

isatuximab + atezolizumab(**)
Anti-CD38 mAb + PD-L1 inhibitor mAb
Solid Tumors

Phase 1

SAR441344
Anti-CD40L mAb
Multiple Sclerosis

SAR443060
RIPK1 inhibitor
Amyotrophic Lateral Sclerosis

SAR443060
RIPK1 inhibitor
Alzheimer's Disease

SAR441000
Cytokine mRNA
Melanoma

REGN5458
Anti BCMA-CD3 bispecific mAb
RRMM

BIVV003
ZFN Gene Editing Technology
Sickle Cell Disease

Next Gen PCV
Pneumococcal Conjugate
Vaccines

(**) Partnered and/or in collaboration – Sanofi may have limited or shared rights on some of these products

Pipeline Movements Since Q3 2018

Removals

Registration

Phase 3

cemiplimab(**) + ipilimumab
PD-1 inhibitor mAb + CTLA4 mAb
1L NSCLC ≥ 50% PDL1+

mavacamten(**)
Myosin inhibitor
Obstructive Hypertrophic Cardiomyopathy

Phase 2

GZ389988
TRKA antagonist
Osteoarthritis

Combination
ferroquine / OZ439(**)
Antimalarial

ALX0171
Anti RSV Nanobody
Respiratory Syncitial Virus

SAR425899
GLP-1/GCG dual agonist
Obesity/Overweight in T2D

mavacamten(**)
Myosin inhibitor
Non-Obstructive Hypertrophic Cardiomyopathy

SAR407899
rho kinase
Microvascular Angina

Phase 1

SAR439794(**)
TLR4 agonist
Peanut Allergy

SAR438335
GLP-1/GIP dual agonist
Type 2 Diabetes

O **REGN3767**
Anti LAG-3 mAb
Advanced Cancers

O **REGN4659**
Anti-CTLA-4 mAb
Cancer

SAR228810(**)
Anti-protofibrillar AB mAb
Alzheimer's Disease

SAR440181(**)
Myosin activation
Dilated Cardiomyopathy

SAR247799
S1P1 agonist
Cardiovascular indication

O **cemiplimab(**) + REGN4659**
PD-1 inhibitor mAb + Anti-CTLA-4 mAb
NSCLC

O **cemiplimab(**) + REGN3767**
PD-1 inhibitor mAb + anti LAG-3 mAb
Advanced Cancers

UshStat®(**)(1)
Myosin 7A gene therapy
Usher Syndrome 1B

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(**) Partnered and/or in collaboration – Sanofi may have limited or shared rights on some of these products
(1) Discontinuation contingent upon identification of out-licensing partner

R&D Pipeline Summary – Total Projects(1)

	Phase 1	Phase 2	Phase 3	Registration	TOTAL
Immuno-inflammation	1	8	7	2	18
Oncology	11	6	7	1	25
Rare Diseases	0	4	3	0	7
Rare Blood Disorders	4	0	2	0	6
Multiple Sclerosis and Neurology	3	2	2	0	7
Diabetes	0	0	4	1	5
Cardiovascular Disease	0	0	1	1	2
Vaccines	3	4	4	0	11
TOTAL	**22**	**24**	**30**	**5**	

46 **35**

81 **Total Projects**

(1) Includes 3 Phase 1 products for which Sanofi has Opt-in rights but has not yet exercised these rights

Expected R&D Milestones

Products	Expected milestones	Timing
Dupixent®	U.S. regulatory decision in Atopic Dermatitis in Adolescent patients	**Q1 2019**
Zynquista™ (sotagliflozin)	U.S. regulatory decision expected in Type 1 Diabetes	**Q1 2019**
dupilumab	U.S. sBLA filing in Nasal Polyposis	**Q1 2019**
Dupixent®	EU regulatory decision in Asthma in Adult/Adolescent patients	**Q2 2019**
Zynquista™ (sotagliflozin)	EU regulatory decision expected in Type 1 Diabetes	**Q2 2019**
Praluent®	EU regulatory decision in CV events reduction ODYSSEY OUTCOMES	**Q2 2019**
Praluent®	U.S. regulatory decision in CV events reduction ODYSSEY OUTCOMES	**Q2 2019**
cemiplimab	EU regulatory decision expected in Advanced Cutaneous Squamous Cell Carcinoma	**Q2 2019**
dupilumab	Start of Phase 2b/3 trial in Chronic Obstructive Pulmonary Disease	**H1 2019**
Dupixent®	EU regulatory decision in Atopic Dermatitis in Adolescent patients	**Q3 2019**
sutimlimab	Expected pivotal trial read-out in Cold Agglutinin Disease	**Q4 2019**
Zynquista™ (sotagliflozin)	Expected pivotal trial read-out in Type 2 Diabetes	**Q4 2019**
Dupixent®	Expected pivotal trial read-out in Atopic Dermatitis in 6-11 years	**Q4 2019**
Olipudase	Expected pivotal trial read-out in Niemann Pick Type B	**Q4 2019**
Isatuximab	Expected pivotal trial read-out in 1-3L RRMM (IKEMA)	**Q1 2020**

Dupilumab (anti-IL4Rα mAb)
Asthma (1/4)

Study	Description	Patients	Design	Endpoints	Status
LIBERTY ASTHMA TRAVERSE LTS12551 NCT02134028	Phase 2/3 Open label extension study long-term safety & tolerability evaluation in patients with asthma who participated in previous studies	2,284 enrolled	• For patients coming from DRI12544, PDY14192, EFC13579, EFC13691 studies, added to current controller medications • Open-label,	• Primary: N and % of patients experiencing any TEAE • Secondary: Safety	• SSD: Jul. 2014 • DE: 2019
Continuation of LIBERTY ASTMA TRAVERSE LPS15023 NCT03620747	Phase 3 Continuation of TRAVERSE evaluating Dupilumab safety in Patients with Asthma (Long term follow-up)	750	• Patients with asthma who completed the treatment period in the previous dupilumab asthma clinical study LTS12551 • Open-label, Single group assignement	• Primary: TEAEs: % of patients reporting TEAs, event rates per 100 patient-year	• SSD: Aug. 2018 • DE: 2021

Dupilumab (anti-IL4Rα mAb)
Asthma (2/4)

Study	Description	Patients	Design	Endpoints	Status
LIBERTY ASTHMA EXCURSION LTS14424 NCT03560466	Phase 3 Long term safety and tolerability (1 year) of dupilumab in pediatric patients with asthma who participated in a previous dupilumab asthma clinical study	540	• Open-label • 1 year of Tx	• Primary: N of patients experiencing any TEAE • Secondary: Severe asthma exacerbation events, change in % predicted FEV1, in absolute FEV1, in FVC, FEF, dupilumab concentrations, anti-dupilumab Ab, eosinophils, Ig, IgE	• SSD: June 2018 • DE: 2026

Dupilumab (anti-IL4Rα mAb)
Asthma (3/4)

Study	Description	Patients	Design	Endpoints	Status
CHILDREN ASTHMA VOYAGE EFC14153 NCT02948959	Phase 3 Evaluation of dupilumab in children (6 to <12 years) with uncontrolled asthma	471	• In children 6 to <12 years of age with uncontrolled persistent asthma • Randomized, Double-blind, Placebo-controlled, parallel group 52 weeks Tx, 12 weeks post Tx	• Primary: Annualized rate of severe exacerbation events during Tx period • Secondary: Safety and tolerability, PROs, Systemic exposure and incidence of anti-drug antibodies, Association between dupilumab Tx and pediatric immune responses to vaccines	• SSD: Jun. 2017 • DE: 2021

Dupilumab (anti-IL4Rα mAb)
Asthma (4/4)

Study	Description	Patients	Design	Endpoints	Status
Persistent Asthma EFC13995 NCT03782532	Phase 3 Efficacy and Safety of dupilumab in patients with Persistent Asthma	486	• In adults and adolescents with a diagnosis of asthma for ≥ 12 months • Randomized, Double-blind, Placebo-controlled, parallel group, • 2 arms: dupilumab and placebo, with in each arm patients with and without oral corticosteroids (OCS) maintenance therapy • Study duration: 40 weeks study including 4 to 5 weeks of screening period, 24 weeks Tx and 12 weeks post Tx	• Primary: change in pre-bronchodilator FEV1 at week 12 for patients without OCS • Secondary: change in FEV1 in overall population, annualized rate of exacerbation events, of LOAC event, of severe exacerbation resulting in hospitalization, time to first exacerbation event, time to first LOAC, change in Asthma Control Questionnaire, asthma symptoms score, nocturnal awakenings, use of daily puffs of rescue medication, Asthma QoL	• SSD: Jan. 2019 • DE: 2021

Dupilumab (anti-IL4Rα mAb)
Atopic Dermatitis (AD) (1/2)

Study	Description	Patients	Design	Endpoints	Status
OLE Pediatrics AD R668-AD-Reg 1434 NCT02612454	Phase 3 A study to assess the long-term safety of dupilumab administered in patients 6 to <18 years of age with AD	765 expected	• For patients having participated in a prior dupilumab study in pediatrics with AD • Open label extension study	• Primary: Incidence and rate of TEAEs • Secondary: SAEs and TEAEs of special interest, % of patients who achieve and maintain remission, EASI-75: % of patients achieving and maintaining at least 75% reduction in EASI score over time, EASI-50: % of patients achieving and maintaining at least 50% reduction in EASI scores over time	• SSD: Oct. 2015 • DE: 2024

Dupilumab (anti-IL4Rα mAb)
Atopic Dermatitis (AD) (2/2)

Study	Description	Patients	Design	Endpoints	Status
LIBERTY AD PRESCHOOL R668-AD-1539 NTC03346434	Phase 2/3 Safety, Pharmacokinetics and Efficacy of Dupilumab in Patients ≥6 Months to <6 Years With Severe Atopic Dermatitis	280	• Part A: Open-label, single-ascending dose, sequential cohort phase 2 study • Part B: Randomized, double-blind, parallel-group, placebo-controlled phase 3 study	• Part A: PK • Part B: Proportion of patients with Investigator's Global Assessment "0" or "1" (on a 5-point scale) at week 16	• SSD: Dec. 2017 • DE: 2021
AD in 6 - 11 Years Old R668-AD-1652 NCT03345914	Phase 3 Efficacy and safety of Dupilumab administered with Topical Corticosteroids in participants ≥6 to <12 years with Severe Atopic Dermatitis	330	• Randomized, Double-blind, Placebo-controlled Study	• Primary: Proportion of patients with Investigator's Global Assessment "0" or "1" (on a 5-point scale) at week 16 • Secondary: Change from baseline to week 16 in Children's Dermatology Life Quality Index, Percent change in EASI score from baseline to week 16, Incidence of serious TEAEs through week 16	• SSD: Jan. 2018 • DE: 2019

Dupilumab (anti-IL4Rα mAb)
Atopic Dermatitis (AD) (3/3)

Study	Description	Patients	Design	Endpoints	Status
Open-Label R668-AD-1225 NCT01949311	Phase 3 Open-Label study of Dupilumab in patients with Atopic Dermatitis	2733 enrolled	• Open label extension study for patients who participated in placebo-controlled dupilumab AD trials. The study primarily evaluates long term safety (adverse events) and immunogenicity. Efficacy parameters are based on IGA, EASI) and the NRS.	• Primary: TEAEs, • Secondary: SAEs and AEs of special interest, % of patients who achieve and maintain remission, EASI-75: % of patients achieving and maintaining at least 75% reduction in EASI score over time, EASI-50: % of patients achieving and maintaining at least 50% reduction in EASI scores over time	• SSD: Oct. 2013 • DE: Last Patient Last Visit: 2022

Dupilumab (anti-IL4Rα mAb)
Grass Immunotherapy

Study	Description	Patients	Design	Endpoints	Status
GRASS R668 – ALG - 16115 NCT03558997	Phase 2 Evaluation of dupilumab as an adjunct for subcutaneous grass immunotherapy to reduce provoked allergic rhinitis symptoms	100	• Patients with history of grass pollen-induced seasonal allergic rhinitis confirmed by SPT with Timothy grass extract and Timothy grass specific IgE, • Randomized, double-blind, placebo-controlled study, • 4 arms: dupilumab + Timothy Grass SCIT; placebo dupilumab + SCIT; dupilumab + placebo SCIT; placebo dupilumab + placebo SCIT; • 16 weeks of Tx	• Primary: Total Nasal Symptom Score (TNSS) after nasal allergen challenge (NAC) with Timothy grass extract, • Secondary: change from baseline in TNSS AUC post NAC over the 1st hour after the challenge across the arms, specific immunoglobulin G4, TEAEs	• SSD: June 2018 • DE: 2019

Dupilumab (anti-IL4Rα mAb)
Eosinophilic Esophagitis (EoE)

Study	Description	Patients	Design	Endpoints	Status
EoE R668 – EE - 1774 NCT03633617	Phase 3 Efficacy and Safety of Dupilumab in Adult and Adolescent patients with Eosinophilic Esophagitis	425	• Patients with documented diagnosis of EoE by endoscopic biopsy, • Randomized, double-blind, parallel assignment, placebo-controlled study, • Part A: dupilumab or placebo (double-blind) for 24 weeks, • Part B: dupilumab dose regimen 1, dupilumab dose regimen 2 or placebo (double-blind) for 24 weeks • Part C: for patients eligible at the end of Part A and Part B, dupilumab dose regimen 1, dupilumab dose regimen 2 (double-blind) for 28 weeks • 12-week follow-up for all patients (eligible and non eligible)	• Primary: Proportion of patients achieving peak esophageal intraepithelial eosinophil count of ≤6 eosinophils per high-power field (eos/hpf), Absolute change in Dysphagia Symptom Questionnaire (DSQ) score • Secondary: Absolute change in EoE endoscopic reference score (EREFS), Percent change in peak esophageal intraepithelial eosinophil count (eos/hpf), Absolute change in EoE Histology Scoring System (EoEHSS), Proportion of patients achieving peak esophageal intraepithelial eosinophil count of ≤15, Proportion of patients achieving peak esophageal intraepithelial eosinophil count of ≤1, Percent change in DSQ, QOL, Absolute change in severity and/or frequency of EoE symptoms other than dysphagia	• SSD: Nov. 2018 • DE: primary completion: 2022, full completion: 2023

Dupilumab (anti-IL4Rα mAb) adjunct to AR101
Peanut Allergy

Study	Description	Patients	Design	Endpoints	Status
Peanut Allergy R668 –ALG - 16114 NCT03682770	Phase 2 Efficacy and Safety of Dupilumab as adjunt to AR101 in Pediatric Subjects with Peanut Allergy	156	• Child 6 to 17 years experiencing dose-limiting symptoms at or before the challenge dose of peanut protein on screening and not experiencing dose-limiting symptoms to placebo • Randomized, double-blind, parallel assignment, placebo-controlled study, • 2 arms: dupilumab adjunct to AR101vs placebo	• Primary: % of subjects who “pass” a double-blind, placebo-controlled food challenge (DBPCFC) with peanut protein at week 28, • Secondary: change in cumulative tolerated dose of peanut protein during DBPBFC, at week 28, % of subjects who « pass » the DBPCFC at week 52 (desensitization maintenance), safety and tolerability, change in peanut-specific IgE (sIgE), IgG4 and IgG4/sIgE ratio	• SSD: Oct. 2018 • DE: primary completion: 2020, full completion: 2021

Sarilumab (anti-IL6 mAb)
Rheumatoid Arthritis (RA)

Study	Description	Patients	Design	Endpoints	Status
SARIL-RA-EXTEND LTS11210 NCT01146652	Phase 3 Long-term evaluation of sarilumab in RA patients	2000	• In patients with RA having participated to previous trials • Multi-center, uncontrolled extension, open-label; up to 1 week screening, at least 264 weeks of open label Tx and up to 516 weeks max., 6 weeks post-Tx	• Primary: N of patients with AE • Secondary: Long term efficacy of sarilumab in patients with RA (ACR20, DAS28, EULAR response)	• SSD: Jun. 2010 • DE: 2020

Sarilumab (anti-IL6 mAb)
Juvenile Idiopathic Arthritis (JIA)

Study	Description	Patients	Design	Endpoints	Status
Polyarticular JIA Children & Adolescents DRI13925 NCT02776735	Phase 2b Dose-finding study of sarilumab in children and adolescents with Polyarticular-course Juvenile Idiopathic Arthritis (pcJIA)	36 in core part, total 60	• In children and adolescents, Aged 2 to 17 years, with pcJIA • Open-label, sequential, ascending, repeated dose-finding Study; 4-week screening, 12-week core Tx, 144-week extension, 6-week post-Tx	• Primary: PK parameters (Up to week 12) • Secondary: PD profile, The efficacy and the safety of sarilumab in patients with pcJIA, Long-term safety of sarilumab in patients with pcJIA	• SSD: Oct. 2016 • DE: 2018 (36 patients CSR); 2020 (60 patients CSR); 2022 (CSR with 3-year extension)
Systemic JIA Children & Adolescents DRI13926 NCT02991469(1)	Phase 2b Dose-finding study of sarilumab in children and adolescents with Systemic Juvenile Idiopathic Arthritis (sJIA)	24 in core part, 48 total	• In children and adolescents, aged 1 to 17 years, with sJIA • Open-label, sequential, ascending, repeated dose finding study, 4-week screening, 12-week coreTx, 144-week extension, 6-week post-Tx	• Primary: PK parameters (Up to week 12) • Secondary: PD profile, efficacy and the safety of sarilumab in patients with sJIA, Long term safety of sarilumab in patients with sJIA	• SSD: Sep. 2018 • DE: 2020 (24 patients CSR); 2022 (48 patients CSR), 2023 (CSR with 3-year extension)

Sarilumab (anti-IL6 mAb)
Giant Cell Arteritis (GCA)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
GCA EFC15068 NCT03600805	Phase 3 Efficacy of sarilumab in combination with corticosteroid in patients with Giant Cell Arteritis	360	• Patients suffering from GCA; new onset active disease or refractory active disease • Randomized, parallel assignment, double-blind, placebo controlled, 2 doses of sarilumab tested vs placebo, in association with prednisone • Study duration per patient: approximately 82 weeks: up to 6 weeks screening, 52-week Tx period, 26-week follow-up period	• Primary: % of patients achieving sustained remission at week 52 • Secondary: components of sustained remission, cumulative corticosteroid dose, time to 1st GCA flare, change in glucocorticoid toxicity index, AEs, PK,	• SSD: Nov. 2018 • DE: primary completion:2021, full completion 2022

Sarilumab (anti-IL6 mAb)
Polymyalgia Rheumatica (PMR)

Study	Description	Patients	Design	Endpoints	Status
PMR EFC15160 NCT03600818	Phase 3 Efficacy of sarilumab in combination with corticosteroid (CS short tapering regimen) in comparison to placebo (CS long tapering regimen) in patients with Polymyalgia Rheumatica	280	• Patients suffering from PMR, • Randomized, parallel assignment, double-blind, placebo controlled, 2 groups: sarilumab + CS, placebo +CS • Study duration per patient: approximately 62 weeks: up to 4 weeks screening, 52-week Tx period, 6-week follow-up period	• Primary: % of patients achieving sustained remission at week 52 • Secondary: components of sustained remission, cumulative corticosteroid dose, time to 1st PMR flare, change in glucocorticoid toxicity index, AEs, PK,	• SSD: Nov. 2018 • DE: 2021

SAR156597 (anti-IL13/IL4 mAb)
Scleroderma

Study	Description	Patients	Design	Endpoints	Status
POC in Scleroderma ACT14604 NCT02921971	Phase 2a Efficacy and safety of SAR156597 in the Tx of Diffuse Cutaneous Systemic Sclerosis (dcSSc)	94	• Randomized, double-blind, Parallel Assignment, placebo-controlled, 4-week screening, 24-week Tx period, 11-week follow-up	• Primary: Change from baseline in mRSS • Secondary: Change from baseline in Health Assessment Questionnaire Disability Index (HAQ-DI), assessed with SHAQ, Change from baseline in respiratory function as measured by observed Forced Vital Capacity Change from baseline in observed Carbon Monoxide Diffusing Lung Capacity (DLco [corrected for hemoglobin])	• SSD: Nov. 2016 • DE : 2019

SAR440340 (Anti-IL33 mAb)
Asthma Combination with dupilumab (1/2)

Study	Description	Patients	Design	Endpoints	Status
Asthma in combination with dupilumab R3500-AS-1633 NCT03112577	Phase 1 Effetcs of SAR440340 dupilumab, combination of both on markers of inflammation after bronchial allergen challenge in patients with Allergic Asthma	38	• Patients with mild allergic asthma for at least 6 months, • Randomized, Placebo –controlled, Parallel Assignment • 5 arms: SAR440340 alone, dupilumab alone, SAR440340 + dupilumab, placebo and fluticasone propionate (active comparator, open label dosing)	• Primary: Difference in bronchial allergen challenge (BAC)-induced changes in sputum inflammatory markers in individuals treated with SAR440340, dupilumab and the combination of both, or placebo [Screening (pre-treatment) to week 4 after treatment initiation] • Secondary: TEAEs (incidence and severity), PK profile, immunogenicity, difference in the BAC-induced changes in sputum inflammatory mRNA signature in individual patients treated with fluticasone	• SSD: July 2017 • DE: 2020 (completion)

SAR440340 (Anti-IL33 mAb)
Asthma Combination with dupilumab (2/2)

Study	Description	Patients	Design	Endpoints	Status
Asthma SA and combination with dupilumab ACT15102 NCT03387852	Phase 2 Efficacy, Safety and Tolerability (POC) of SAR440340 and the coadministration with dupilumab in patients with Moderate-to-severe Asthma, Not Well Controlled on Inhaled Corticosteroid (ICS) Plus Long-acting β2 Adrenergic Agonist (LABA) Therapy	297	• Adults patient with a physician diagnosis of asthma for at least 12 months, • Randomized, Double-blind, Placebo-controlled, Parallel Group, with fluticasone w/wo salmeterol • Arm 1: SAR440340 monotherapy • Arm 2 : dupilumab monotherapy • Arm 3: coadministration of SAR440340 and dupilumab • Arm 4: placebo • Ttmt every 2 weeks for 12 weeks • Total duration for one patient: appr. 36 weeks, including 4 weeks screening,12 weeks ttmt and 20 weeks post-ttmt	• Primary: LOAC (lost of asthma control) events • Secondary: change in FEV1 (forced expiratory volume 1)	• SSD: Mar. 2018 • DE: 2019

SAR440340 (Anti-IL33 mAb)
COPD

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
POC in COPD ACT15104 NCT03546907	Phase 2 Efficacy, Safety and Tolerability (POC) of SAR440340 in patients with moderate-to-severe COPD	340	• Adults patients with a diagnosis of moderate-to-severe COPD for at least 1 year • Randomized, Double-blind, Placebo-controlled, on top of standards of care • Arm 1: SAR440340 • Arm 2 : placebo • Total duration for one patient: 46 to 76 weeks including 10 days to 4 weeks of screening, 24 to 52 weeks Tx period and 20 weeks post IMP Tx period	• Primary: AECOPD (Acute Exacerbations in COPD) • Secondary: average change in pre-bronchodilator FEV1 (forced expiratory volume 1), time to 1st COPD exacerbations, AEs, change in post-bronchodilator FEV1	• SSD: Jul. 2018 • DE: 2020

SAR440340 (Anti-IL33 mAb)
Atopic Dermatitis, Combination with dupilumab (1/2)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
AD R3500-AD-1798 NCT03736967	Phase 2a Efficacy and Safety of SAR440340 Monotherapy and in Combination with Dupilumab in patients with moderate-to-severe Atopic Dermatitis (AD)	280	• Patients with chronic AD present for at least 3 years • Randomized, Double-blind, Placebo-controlled, Parallel-Group, • 4 Arms: SAR440340, dupilumab, combination SAR440340 + dupilumab, placebo	• Primary: Eczema Area and Severity Index (EASI) % of change • Secondary: % of patients with EASI ≥ 50% improvement , % of patients with EASI ≥ 75% improvement, % of patients with EASI ≥ 90% improvement, absolute change in EASI scores, Investor's Global Assessment (IGA), Pruritus Numerical Rating Scale (NRS), SCORing Atopic Dermatitis (SCORAD), SAR440340 serum concentration and antibodies	• SSD: Nov. 2018 • DE: 2020

SAR440340 (Anti-IL33 mAb)
Atopic Dermatitis (2/2)

Study	Description	Patients	Design	Endpoints	Status
DR in AD R3500-AD-1805 NCT03738423	Phase 2b Efficacy, Safety and Pharmacokinetics of SAR440340 in patients with moderate-to-severe Atopic Dermatitis (AD)	300	• Patients with chronic AD present for at least 3 years • Randomized, Double-blind, Placebo-controlled, Parallel-Group, Dose-Ranging study • 5 Arms: 4 SAR440340 doses and placebo	• Primary: Eczema Area and Severity Index (EASI) % of change • Secondary: % of patients with EASI ≥ 50% improvement , % of patients with EASI ≥ 75% improvement, % of patients with EASI ≥ 90% improvement, absolute change in EASI scores, Investor's Global Assessment (IGA), Pruritus Numerical Rating Scale (NRS), SCORing Atopic Dermatitis (SCORAD), SAR440340 serum concentration and antibodies	• SSD: Nov. 2018 • DE: 2020

Isatuximab (anti-CD38 mAb)
Hematological Malignancies (HM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
CD38+HM TED10893 NCT01084252	Phase1/2 Dose escalation and efficacy study of isatuximab in patients with selected CD38+ HM	351 (enrollment completed)	• Phase 1: MTD • Phase 2: Stage 1: isatuximab activity at different doses/schedules and to select dose and regimen as single agent or in combination with dexamethasone Stage 2: activity at the selected dose/schedule from stage1, as single agent (ISA arm) and in combination with dexamethasone (ISAdex arm) • Randomized, Open-label, Parallel assignment	• Primary: DLT, ORR • Secondary: DOR, PFS, OS, Immune Response	• SSD: Jun. 2010 • DE: 2019

Isatuximab (anti-CD38 mAb)
Hematological Malignancies (HM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
CD38+HM TED15085 NCT03733717	Phase1 Pharmacokinetcis Safety and Preliminary Efficacy of isatuximab in Chinese Patients with Relapsed and/or Refractory MM	20	• In Patients with known diagnosis of symptomatic multiple myeloma, • Open-label, Single Group assignment • Isatuximab every week in Cycle 1 (4 weeks) followed by every 2 weeks (Q2W) in subsequent cycles	• Primary: Pharmacokinetics • Secondary: Aes, ORR, DOR, TTP, PFS, OS, immunogenicity	• SSD: Oct. 2018 • DE: 1st data: 2020; Full completion 2022

Isatuximab (anti-CD38 mAb)
Multiple Myeloma (MM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
Lenalidomide Combination **RRMM** TCD11863 NCT01749969	Phase 1b Isatuximab, in Combination With lenalidomide and dexamethasone for the Tx of Relapsed or Refractory MM	57 (enrollment completed)	• Patients with diagnosis of MM and documentation of at least 2 prior therapies (induction therapy, autologous stem cell transplant, consolidation and maintenance therapy is considered one prior therapy) • Open-label, Parallel assignment • Isatuximab (escalating doses) + lenalidomide + dexamethasone • Total duration for one patient: up to 21 days screening, at least 4 weeks Tx, up to 60 days follow-up	• Primary: N of patients with AE • Secondary: ORR, PFS, PK, PD, Immunogenicity	• SSD: Feb. 2013 • DE: 2019

Isatuximab (anti-CD38 mAb)
Multiple Myeloma (MM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
Pomalidomide Combination **RRMM** TCD14079 NCT02283775	Phase 1b Isatuximab, in combination with pomalidomide and dexamethasone for the Tx of Relapsed/Refractory MM	92	• Patients previously diagnosed with MM based on standard criteria and currently require Tx because MM has relapsed following a response • Open-label, Parallel assignment • Isatuximab + pomalidomide + dexamethasone • Part A, doses ranging for isatuximab, (5mg/kg, 10mg/kg, 20mg/kg); Part B isatuximab (10mg/kg) from a fixed infusion volume	• Primary: DLTs, N of patients with AE • Secondary: ORR, PK, Immunogenicity, DOR, CB	• SSD: May 2015 • DE: 2020 1st set of data 2019 (included in Isatuximab BLA)

Isatuximab (anti-CD38 mAb)
Multiple Myeloma (MM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
Bortezomib Combination **NDMM** TCD13983 NCT02513186	Phase 1 Isatuximab in combination with bortezomib - based regimens in adult patients with newly diagnosed MM non eligible for transplantation	44	• Patients with a diagnosis of MM with evidence of measurable disease, having received prior Tx with an IMiD and with at least 3 prior lines of therapy • Open-label, Single Group assignment • Isatuximab (escalating dose) + bortezomib + cyclophosphamide + dexamethasone: VCDI cohort (3-week screening, 50-week duration for induction and then up to disease progression, or unacceptable AEs + follow-up) • Isatuximab + bortezomib + dexamethasone + lenalidomide: VRDI cohort to begin after VCDI completion (4-week screening, 24-week duration for induction and then up to disease progression, or unacceptable AEs, + follow-up)	• Primary: DLTs/VCDI For both VCDI & VRDI: ORR, CR • Secondary: N of patients with AE, and significant changes in lab tests, PK, DOR	• SSD: Sep. 2015 • DE: 1st set of data: 2019; next 2020, Full completion: 2023

Isatuximab (anti-CD38 mAb)
Multiple Myeloma (MM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
RRMM TED14154 NCT02514668	Phase 1 Safety, PK and Efficacy of isatuximab in patients with Relapsed/Refractory MM	58 (enrollment completed)	• Patients with a diagnosis of MM with evidence of measurable disease and with evidence of disease progression • Open-label, Single Group assignment, isatuximab (escalating doses) • Total duration for one patient: up to 21 days screening, Tx period up to disease progression or AEs , 60- day follow-up at least	• Primary: Part A: DLTs, N of patients with AE; Part B: ORR • Secondary: PK, N of patients with AEs, DOR, CB, PFS, Immunogenicity	• SSD: Sep. 2015 • DE: 2019

Isatuximab (anti-CD38 mAb)
Multiple Myeloma (MM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
ISLANDS (Japanese Patients) **RRMM** TED14095 NCT02812706	Phase 1 Phase 2 Isatuximab single-agent in Japanese patients with Relapsed and Refractory MM	36 (enrollment completed)	• Patients with a diagnosis of symptomatic MM, having received at least 3 prior lines of therapy OR whose disease is double refractory to an IMiD and a PI • Open-label, Single Group assignment, isatuximab monotherapy • Total duration for one patient: up to 21-day screening, Tx period up to disease progression or unacceptable AEs, post-Tx follow-up	• Primary: Phase 1: DLTs Phase 2: ORR • Secondary: N of patients with AE, CB, OS, PFS, DOR, TTR, PK, PD, Immunogenicity	• SSD: Sep. 2016 • DE: primary completion 2018; full completion 2019

Isatuximab (anti-CD38 mAb)
Multiple Myeloma (MM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
Cemiplimab Combination **RRMM** TCD14906 NCT03194867	Phase 1 Phase 2 Safety, PK and Efficacy of isatuximab in combination with cemiplimab in patients with Relapsed/Refractory MM	108	• Patients with a diagnosis MM with evidence of measurable disease, having received prior Tx with an IMiD and with at least 3 prior lines of therapy • Randomized, Open-label, Parallel Assignment • Isatuximab + cemiplimab • 3 Arms: Isa +cemi regimen 1; isa + cemi regimen 2; isa alone • Total duration for one patient: up to 21-day screening, Tx period up to disease progression or unacceptable AEs, 3-month post-Tx follow-up. Cycle duration 28 days	• Primary: DLTs, N of patients with AE, ORR • Secondary: CB, DOR, TTR, PFS, OS, PK, Immunogenicity (isatuximab and cemiplimab)	• SSD: Feb. 2018 • DE: Primary: 2019, Next: 2021, Full completion :2023

Isatuximab (anti-CD38 mAb)
Multiple Myeloma (MM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
ICARIA-MM RRMM EFC14335 NCT02990338	Phase 3 Isatuximab, pomalidomide, and dexamethasone to pomalidomide and dexamethasone in Refractory or Relapsed and RRMM	307 (enrollment completed)	• Isatuximab in combination with pomalidomide and low-dose dexamethasone, compared to pomalidomide and low-dose dexamethasone in patients with RRMM • Randomized, Open-label, Parallel assignment	• Primary: PFS • Secondary: ORR, OS, TTP, PFS, DOR	• SSD: Jan. 2017 • DE (1st Part)(1): 2019; full completion 2020

(1) Final Data Collection date for primary outcome measure

Isatuximab (anti-CD38 mAb)
Multiple Myeloma (MM)

Study	Description	Patients	Design	Endpoints	Status
IKEMA RRMM EFC15246 NCT03275285	Phase 3 Isatuximab combined with carfilzomib and dexamethasone vs. carfilzomib with dexamethasone in patients With Relapse and/or Refractory MM previously treated with 1 to 3 prior lines	300	• Patients with MM previously treated with prior 1 to 3 lines and with measurable serum M-protein (≥ 0.5 g/dL) and/or urine M-protein (≥ 200 mg/24 hours) • Randomized, Open-label, Parallel assignment, 2-arm: (a) isatuximab +carfilzomib+dexamethasone, (b) carfilzomib+dexamethasone	• Primary: PFS • Secondary: ORR, % of patients with CR, and VGPR, OS, TTP, Second PFS, DOR, AE, PK, Immunogenicity	• SSD: Oct. 2017 • DE: Primary: 2020, Next: 2021, Full completion: 2023

Isatuximab (anti-CD38 mAb)
Multiple Myeloma (MM) Ti

Study	Description	Patients	Design	Endpoints	Status
IMROZ NDMM Ti EFC12522 NCT03319667	Phase 3 Isatuximab in combination with bortezomib (Velcade®), lenalidomide (Revlimid®) and dexamethasone vs. bortezomib, lenalidomide and dexamethasone in patients with newly diagnosed MM not eligible for transplant	440	• Newly diagnosed MM not eligible for transplant due to age (≥ 65 years) or patients < 65 years with comorbidities impacting possibility of transplant • Randomized, Open-label, Parallel assignment • IVRd arm (Isatuximab/bortezomib/lenalidomide /dexamethasone) • VRd arm (Bortezomib/lenalidomide /dexamethasone) • Ird crossover arm (Isatuximab/lenalidomide/ dexamethasone) • Total duration for each patient: screening period up to 4 weeks, induction period of 24 weeks, continuous Tx period and crossover when applicable	• Primary: PFS • Secondary: ORR, % of patients with CR, and VGPR, % of patients with MRD (Minimal Residual Disease) negative, OS, TTP, DOR, PFS on next line of therapy (PFS2), AE, PK, Immunogenicity, QOL	• SSD: 2017 • DE: Primary: 2021, Next: 2023, Full completion: 2025

Isatuximab (anti-CD38 mAb)
Multiple Myeloma (MM) Te

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
NDMM Te GMMG HD7 NCT03617731	Phase 3 Effect of Isatuximab in induction therapy with lenalidomide - bortezomib - dexamethasone (RVd) and lenalidomide maintenance Tx in patients with newly diagnosed myeloma	662	• Confirmed diagnosis of untreated multiple myeloma requiring systemic therapy and eligible for high dose therapy and autologous stem cell transplantation • Randomized, Open-label, Parallel assignment • Induction: 2 arms: IA: 3 cycles RVd, IB: 3 cycles RVd + isatuximab • After induction therapy autologous stem cell transplantation performed, • Maintenance: 2 arms: IIA lenalidomide for 3 years; IIB: lenalidomide + isatuximab for 3years	• Primary: MRD negative after induction Tx, PFS after 2nd randomization (IIA & IIB) • Secondary: PFS, OS, CR, MRD, Best response to Tx, PFS after next line of therapy from 2nd randomization, AEs, QOL, PK, immunogenicity	• SSD: Oct. 2018 • DE: 1st part Q4 2021 for MRD negativity after induction and 2023 (PFS IA); full completion 2025

Isatuximab (anti-CD38 mAb) combination cemiplimab (PD-1 inhibitor) – Advanced Malignancies

Study	Description	Patients	Design	Endpoints	Status
Advanced Malignancies ACT15319 NCT03367819	Phase 1/2 Safety and tolerability of Isatuximab in combination with cemiplimab in patients with metastatic castration-resistant prostate cancer (mCRPC) or patients with non-small cell lung cancer (NSCLC)	132	• In Patients with metastatic, castration-resistant prostate cancer (mCRPC) who are naïve to anti-programmed cell death-1 (PD-1)/programmed cell death-ligand 1 (PDL-1)-containing therapy, or non-small cell lung cancer (NSCLC) who progressed on anti-PD-1/PDL-1-containing therapy, • Randomized, Open-Label, Parallel Assignment • Isatuximab alone or in combination with cemiplimab • Total duration per patient up to 28 months including 28 days screening period, , up to 24 months ttmt period and 3 months safety FU	• Primary: Safety, tolerability, RR • Secondary: Immunogenicity (isa and cemi), PK, tumor burden change, DR, PFS, Disease Control Rate	• SSD: Jan. 2018 • DE: Primary:2021, Full completion: 2022

Isatuximab (anti-CD38 mAb) combination cemiplimab (PD-1 inhibitor) – Lymphoma

Study	Description	Patients	Design	Endpoints	Status
Advanced Malignancies ACT15320 NCT03769181	Phase 1/2 Safety, Preliminary Efficacy and Pharmacokinetics of Isatuximab in combination with cemiplimab in patients with Lymphoma	118	• In Patients with Lymphoma: *Cohort A1*: classic Hodgkin'Lymphoma (cHL) anti-PD-1/PD-L1 inhibitor naïve, *Cohort A2*: cHL) anti-PD-1/PD-L1 inhibitor progressor *Cohort B*: diffuse large B-cell Lymphoma (DLBCL) *Cohort C*: peripheral T-cell Lymphoma (PTCL) • Non-Randomized, Open-Label, Parallel Assignment • Isatuximab in combination with cemiplimab	• Primary: *Phase 1*: DLTs, recommended Phase 2 dose (RP2D), *Phase 2*: Cohort A1: Complete Remission Rate (CRR); Cohort A2 RR • Secondary: Aes, SAEs, PK, tumor burden, disease control rate, DR, PFS	• SSD: Jan. 2019 • DE: Primary: 2021, Full completion: 2022

Isatuximab (anti-CD38 mAb) combination atezolizumab (PD-1 inhibitor) – Advanced Malignancies

Study	Description	Patients	Design	Endpoints	Status
Advanced Malignancies ACT15377 NCT03637764	Phase 1/2 Safety, Preliminary Efficacy and Pharmacokinetics of Isatuximab monotherapy or in combination with Atezolizumab in patients with Advanced Malignancies	220	• In Patients with a known diagnosis of either unresectable HCC, platinum-refractory /recurrent /metastatic SCCHN, platinum-resistant/refractory EOC with evidence of measurable disease or recurrent GBM, • Non-Randomized, Open-Label, Parallel Assignment, • Isatuximab alone or in combination with atezolizumab,	• Primary: DLTs, AEs, MTD, Recommended Phase 2 dose, RR, PFS, • Secondary: immunogenicity (Isatuximab and atezolizumab), tumor burden change, disease control rate, DR, PFS, RR, PK,	• SSD: Aug. 2018 • DE: Primary:2021, Full completion: 2022

Isatuximab (anti-CD38 mAb) combination atezolizumab (PD-1 inhibitor) – Solid Tumors

Study	Description	Patients	Design	Endpoints	Status
Umbrella Trial For sanofi: ACT16241 NCT03555149	Phase 1b/2 Efficacy and Safety, of isatuximab in combination with atezolizumab in patients with Metastatic Colorectal Cancer	160	• Umbrella study, Randomized, Open-Label, Parallel Assignment, • Isatuximab in combination with atezolizumab, • Patients will receive Tx until unacceptable toxicity or loss of clinical benefit as confirmed by disease progression or lack of continued benefit as determined by the investigator	• Primary: ORR, AEs • Secondary: PFS, OS, DOR, % of patients alive at Month 6, DCR, immunogenicity,	• SSD: Sep. 2018 • DE: 2021

Cemiplimab (PD-1 inhibitor)
Advanced Malignancies (AM)

Study	Description	Patients	Design	Endpoints	Status
AM R2810-ONC-1423 NCT02383212	Phase 1 A first-in-human study of repeat dosing with cemiplimab, as single therapy and in combination with other Anti-Cancer therapies in patients with AM	398	• Non-Randomized, Open-label, Parallel assignment, ascending-dose • Monotherapy, cemiplimab alone • Dual combination: cemiplimab in combination with hypofractionated radiotherapy or with cyclophosphamide or with docetaxel • Triple combination: cemiplimab with hypofractionated radiotherapy plus cyclophosphamide, or hypofractionated radiotherapy plus GM-CSF or carboplatin plus paclitaxel or carboplatin plus pemetrexed or carboplatin plus docetaxel • Quadruple combination: cemiplimab with hypofractionated radiotherapy plus GM-CSF plus cyclophosphamide	• Primary: TEAE, Incidence of abnormal laboratory findings, N of participants with DLT • Secondary, RECIST as measured by CT or MRI, Immune-Related Response	• SSD: Jan. 2015 • DE: 2020

Cemiplimab (PD-1 inhibitor)
Advanced Malignancies (AM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
PK in Japanese patients AM R2810-ONC-1622 NCT03233139	Phase 1 To investigate the safety and PKs of cemiplimab in Japanese patients with AM	81	• Part 1: Histologically or cytologically confirmed diagnosis of malignancy with no alternative standard-of-care therapeutic option • Part 2: Histologically or cytologically documented squamous or non-squamous NSCLC with stage IIIB or stage IV disease who received no prior systematic ttmt for recurrent or metastatic NSCLC. In Part 2 patients must have available archival or newly obtained formalin-fixed tumor tissue from a metastatic/recurrent site, which has not previously been irradiated. • Sequantial assignment, Open-label, non-randomized • 3 arms: Part 1: cemiplimab; Part 2/ cohort A: cemiplimab; Part 2/ cohort B: cemiplimab + ipilimumab + platinum doublet chemotherapy	• Primary: TEAEs cemiplimab PK parameters • Secondary: Immunogenicity against cemiplimab, ORR, DOR	• SSD: Sep. 2017 • DE: primary completion 2019; full completion 2023

Cemiplimab (PD-1 inhibitor) Combination REGN3767 Advanced Malignancies (AM)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
R3767-ONC-1613 NCT03005782	Phase 1 To investigate the safety and PKs of REGN3767 (anti LAG-3 mAb) to determine the recommended Phase 2 dose (RP2D) as monotherapy and in combination with cemiplimab in patients with advanced malignancies	546	• Histologically or cytologically confirmed diagnosis of malignancy with no alternative standard-of-care therapeutic option • Non-randomized, Parallel Group assignment, Open-label • Group A: REGN3767, 4 sequential dose cohorts, each cohort receiving 1 of 3 ascending dose levels. 1 tumor-specific cohort treated with the RP2D during dose expansion • Group B: REGN3767+cemiplimab, same design; 9 tumor-specific cohorts treated with RP2D	• Primary: DLTs, PK parameters, AEs, SAEs, death and lab. abnormalities, response rate • Secondary: Response rate, duration of response, disease control rate, PFS, Aes, SAEs, death, lab. abnormalities immunogenicity	• SSD: Nov. 2016 • DE: Primary completion 2021, full completion 2022

Cemiplimab (PD-1 inhibitor)
Melanoma

Study	Description	Patients	Design	Endpoints	Status
Biomarkers Melanoma R2810-ONC-1606 NCT03002376	Phase 1 Exploratory Tumor Biopsy-driven study to understand the relationship between biomarkers and clinical response in Melanoma patients receiving cemiplimab	50	• For Histologically confirmed diagnosis of stage III (unresectable) or stage IV cutaneous melanoma (non-acral lentiginous) with at least 1 lesion that is measurable by RECIST 1.1 criteria and accessible for biopsies • Non-Randomized, Open-label, Parallel assignment • Group 1: Patients with metastatic CSCC: to distant sites or lymph nodes. cemiplimab administered intravenously every 2 weeks • Group 2: Patients with unresectable locally advanced CSCC. cemiplimab administered intravenously every 2 weeks • Group 3: Patients with metastatic CSCC, to distant sites or lymph nodes. cemiplimab administered intravenously every 3 weeks	• Primary: Correlation between changes in the tumor microenvironment and the change in tumor volume following cemiplimab Tx • Secondary: Correlation between baseline tumor characteristics and the change in tumor volume following Tx, cemiplimab serum concentrations, antibodies levels, PFS, ORR	• SSD: Apr. 2017 • DE: Full completion: 2019

(1) Final Data Collection date for primary outcome measure

Cemiplimab (PD-1 inhibitor)
Head and Neck

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
Biomarkers Head & Neck R2810-ONC-1655 NCT03198130	Phase 1 Exploratory Tumor Biopsy-driven study to understand the relationship between biomarkers and clinical response in Immunomodulatory Treatment-Naïve patients with Recurrent and/or Metastatic Squamous Cell Carcinoma of Head and Neck receiving cemiplimab	30	• For Histologically confirmed diagnosis recurrent and/or metastatic SCCHN (squamous cell carcinoma of the head and neck) with no curative options with at least 1 lesion that is measurable by Response Evaluation Criteria in Solid Tumors (RECIST) • Open-label, Single Group Assignment	• Primary: Correlation between changes in the tumor microenvironment and the change in tumor volume following cemiplimab Tx • Secondary: Correlation between baseline tumor characteristics and the change in tumor volume following Tx, ORR, PFS, TAES, cemiplimab serum concentration, anti-cemiplimab antibodies level	• SSD: Jul. 2017 • DE (1st Part) (1): 2019; full completion 2020

(1) Final Data Collection date for primary outcome measure

Cemiplimab (PD-1 inhibitor)
Cutaneous Squamous Cell Carcinoma (CSCC)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
Advanced CSCC R2810-ONC-1540 NCT02760498	Phase 2 Cemiplimab monotherapy for patients with metastatic (nodal or distant) CSCC (Groups 1 and 3) or with unresectable locally advanced CSCC (Group 2)	266	• Non-Randomized, Open-label, Parallel assignment • Group 1: Patients with metastatic CSCC: to distant sites or lymph nodes cemiplimab administered intravenously every 2 weeks • Group 2: Patients with unresectable locally advanced CSCC. cemiplimab administered intravenously every 2 weeks • Group 3: Patients with metastatic CSCC: to distant sites or lymph nodes, cemiplimab administered intravenously every 3 weeks • Group 4: Patients with advanced CSCC , metastatic (nodal or distant) or unresectable locally advanced , cemiplimab administered every 4 weeks	• Primary: ORR (96 weeks), Groups 1,3 and 4: RECIST version 1.1 will be used to determine ORR, Group 2 and 4: Clinical response criteria will be used to determine ORR • Secondary: Investigator Assessments of ORR, DOR, PFS, OS, CRR	• SSD: May 2016 • DE: Primary:2020; Full completion 2021
Expanded Access CSCC R2810-ONC-17103 NCT03492489	Expanded Access Tx IND/Protocol Provide access to cemiplimab to patients with mCSCC or locally advanced CSCC, who are not candidate for surgery prior to cemiplimab being commercially available	Intermediate-size Population			

Cemiplimab (PD-1 inhibitor)
Basal Cell Carcinoma (BCC)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
BCC R2810-ONC-1620 NCT03132636	Phase 2 Cemiplimab in patients with Advanced BCC who experienced progression of disease on Hedgehog Pathway Inhibitor Therapy, or were intolerant of Prior Hedgehog Pathway Inhibitor Therapy	137	• Patients with confirmed diagnosis of invasive BCC • Non-Randomized, Open-label, Parallel assignment • Group 1: Patients with metastatic BCC • Group 2: Patients with unresectable locally advanced BCC	• Primary: ORR for mBCC measured by RECIST version 1.1 ORR for unresectable locally advanced BCC measured by Composite Response Criteria • Secondary: DOR, CR, PFS, OS, TEAEs, PK, immunogenicity	• SSD: July 2017 • DE: Primary: 2020, Full completion 2021

Cemiplimab (PD-1 inhibitor)
Non-Small Cell Lung Cancer (NSCLC)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
mNSCLC R2810-ONC-1624 NCT03088540	Phase 3 First-line Tx in patients with advanced or metastatic NSCLC whose tumors express PD-L1, vs. Platinum Based Chemotherapy	700	• For histologically or cytologically documented squamous or non squamous NSCLC with stage IIIB or stage IIIC who are not candidates for Tx with definitive chemoradiation or patients with stage IV disease who received no prior systemic Tx for recurrent or metastatic NSCLC • Randomized, Open-label, Cross-over assignment • Active Comparator: Standard-of-care chemotherapy: paclitaxel + cisplatin OR paclitaxel + carboplatin OR gemcitabine + cisplatin or gemcitabine + carboplatin OR Pemetrexed + cisplatin followed by optional pemetrexed maintenance OR pemetrexed + carboplatin followed by optional pemetrexed maintenance	• Primary: PFS as assessed by a blinded Independent review committee using RECIST 1.1 • Secondary: OS, Objective response rates, BOR, DOR	• SSD: May 2017 • DE: 2022

Cemiplimab (PD-1 inhibitor)
Non-Small Cell Lung Cancer (NSCLC)

Study	Description	Patients	Design	Endpoints	Status
mNSCLC R2810-ONC-16113 NCT03409614	Phase 3 Combination of cemiplimab, ipilimumab and Platinum-based Doublet Chemotherapy in 1st Line Tx of patients with advanced or metastatic NSCLC with tumors expressing PD-L1<50%	690	• For histologically or cytologically documented squamous or non squamous NSCLC with stage IIIB or IIIC disease who are not candidates for Tx with definitive concurrent chemoradiation or stage IV disease who received no prior systemic Tx for recurrent or metastatic NSCLC • Randomized, Open-label, Parallel assignment • Arm 1: Standard of care Platinum-based doublet chemotherapy • Arm 2: cemiplimab + Platinum-based doublet chemotherapy • Arm 3: cemiplimab + abbreviated chemotherapy + ipilimumab	• Primary: PFS as assessed by a blinded Independent review committee using RECIST 1.1 • Secondary: OS, ORR, TEAEs, DLTs, SAEs, death, lab. abnormalities, OS, QoL	• SSD: Mar. 2018 • DE: 2022

Protocol amendment in preparation

Cemiplimab (PD-1 inhibitor)
Non-Small Cell Lung Cancer (NSCLC)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
mNSCLC R2810-ONC-16111 NCT03515629	Phase 3 Combination of cemiplimab, Platinum-based Doublet Chemotherapy, and ipilimumab vs pembrolizumab in in 1st Line Tx of patients with advanced or metastatic NSCLC with tumors expressing PD-L1 ≥ 50%	585*	• For histologically or cytologically documented squamous or non squamous NSCLC with stage IIIB or stage IV disease who received no prior systemic Tx for recurrent or metastatic NSCLC • Randomized, Open-label, Parallel assignment • Arm 1: pembrolizumab • Arm 2: cemiplimab + ipilimumab • Arm 3: cemiplimab + chemotherapy + ipilimumab	• Primary: PFS as assessed by a blinded Independent review committee using RECIST 1.1 • Secondary: OS, ORR, TEAEs, DLTs, SAEs, death, lab. abnormalities, OS, QoL	• SSD: June 2018 • DE: 2023

*: to be adjusted; study ongoing with the patients included but recruitment stopped

Cemiplimab (PD-1 inhibitor)
Non-Small Cell Lung Cancer (NSCLC)

Study	Description	Patients	Design	Endpoints	Status
mNSCLC R2810-ONC-1763 NCT03430063	Phase 2 Combination of standard and High dose of cemiplimab and ipilimumab in 2nd Line Tx of patients with Advanced NSCLC	252*	• For histologically or cytologically documented squamous or non squamous NSCLC with stage IIIB or IIIC and not candidates for definitive chemoradiation or stage IV. Patients must have PD after receiving one prior line of chemotherapy Tx for advanced NSCLC, • Randomized, Open-label, Parallel assignment • Arm 1: cemiplimab standard dose • Arm 2: cemiplimab + ipilimumab standard doses • Arm 3: cemiplimab High dose	• Primary: ORR • Secondary: OS, PFS, TEAEs, SAEs, death, lab. abnormalities	• SSD: May 2018 • DE: 2022

*: to be adjusted; study ongoing with the patients included but recruitment stopped

Cemiplimab (PD-1 inhibitor) Combination REGN4659 Non-Small Cell Lung Cancer (NSCLC)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
mNSCLC R4659-ONC-1795 NCT03580694	Phase 1 Cemiplimab in combination with REGN4959 in the Tx of patients with advanced or mNSCLC	134	• For histologically or cytologically documented squamous or non squamous NSCLC with stage IIIB or stage IV who received no prior systemic Tx for recurrent or mNSCLC; with expression of PDL-1in ≥ 50% of tumors cells • Non-Randomized, Open-label, Parallel assignment • Arm 1: cemiplimab • Arm 2: cemiplimab + REGN4659	• Primary: DLTs, TEAEs, immune-related AEs, SAEs, deaths, lab. abnormalities, ORR, PK of both products • Secondary: ORR, BOR, DOR, disease control rate, PFS, OS,	• SSD: June 2018 • DE: 2021

Cemiplimab (PD-1 inhibitor)
Cervical cancer (CC)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
CC R2810-ONC-1676 NCT03257267	Phase 3 Cemiplimab vs. therapy of Investigator Choice chemotherapy in Recurrent or Metastatic Platinum-Refractory CC	436	• Patients with recurrent or metastatic platinum-refractory CC for which there is no curative intent option, • Randomized, Open-label, Parallel assignment, Tx cycle 6 weeks, Planned Tx for up to 96 weeks • 2 arms: cemiplimab and Investigator Choice (IC) chemotherapy	• Primary: OS • Secondary: PFS, ORR, DOR, QOL	• SSD: Oct. 2017 • DE: Primary: 2020; Next 2022; Full completion 2023

Cemiplimab (PD-1 inhibitor) Combination REGN4018
Ovarian cancer (OC)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
CC R4018-ONC-1721 NCT03564340	Phase 1/2 REGN4018 alone or in combination with cemiplimab in patients with Platinum-Resistant Ovarian Cancer	264	• Histologically or cytologically confirmed diagnosis of advanced, epithelial ovarian (except carcinosarcoma), primary peritoneal, or fallopian tube cancer with CA-125 ≥ 2 xULN, progression or relapse within 6 months of the most recent Tx with Platinum-containing chemotherapy, documented progression and no standard therapy options • Non- Randomized, Open-label, Parallel assignment, • Arm 1: REGN4018 • Arm 2: REGN4018 + cemiplimab	• Primary: DLTs, TEAEs, SAEs, deaths, lab abnormalities, drugs serum concentrations, ORR • Secondary: BOR, DOR, disease control, PFS, CA-125	• SSD: May 2018 • DE: 2022

SAR439459 (TGFß inhibitor mAb)
Advanced Solid Tumors (AST)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
AST **Monotherapy and combination with cemiplimab** TCD14678 NCT03192345	Phase 1/1b Safety, PK, PD and Anti-tumor activity of SAR439459 Monotherapy and in combination with cemiplimab in adult patients with AST	130	• Patients with histologically confirmed, advanced unresectable or metastatic solid tumor • Open-label, Parallel assignment • Part 1A: SAR439459 monotherapy escalating doses • Part 2A: SAR439459 monotherapy with the previously recommended dose • Part 1B: SAR439459 escalating dose + cemiplimab standard dose • Part 2B: SAR439459 at previously recommended dose + cemiplimab standard dose • Escalation periods non randomized followed by expansion periods randomized	• Primary: incidence of DLTs (Part 1), ORR (Part 2) • Secondary: Safety profile, Immunogenicity, PK, PFS (Part 2), TTP (Part 2)	• SSD: Jun. 2017 • DE: 2021

SAR408701 (maytansin loaded anti-CEACAM5 mAb) Advanced Solid Tumors (AST) 1/2

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
First-in-Human TED13751 NCT02187848	Phase 1 Phase 2 Safety, PK and antitumor activity of SAR408701 in patients with AST	313	• Patients with locally advanced or metastatic solid malignant tumor • Non-Randomized, Open-label, Parallel assignment • Arm 1 : SAR408701 monotherapy escalating cohorts • Arm 2: SAR408701 expansion cohort in CRC with MTD previously defined • Arm 3: SAR408701 expansion cohort in non-squamous NSCLC high expresser patients (CEACAM5 >50% of tumor cells ≥ 2+ intensity) at MTD • Arm 4: SAR408701 expansion cohort gastric adenocarcinoma at MTD • Arm 5: SAR408701 loading dose at first cycle followed by MTD • Arm 6: SAR408701 expansion cohort in non-squamous NSCLC patients (Lung bis) with 50%> CEACAM5 >1% of tumors cells ≥ 2+ intensity, at MTD • Arm 7: SAR408701 expansion cohort SCLC at MTD	• Primary: MTD, Anti-tumor response RECIST • Secondary: Overall Safety, Immunogenicity, PK, duration of response, time to progression	• SSD: Sep. 2014 • DE: 2021

SAR408701 (maytansin loaded anti-CEACAM5 mAb) Advanced Solid Tumors (AST) 2/2

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
Japanese patients **Monotherapy** TCD15054 NCT03324113	Phase 1 Safety and PK of SAR408701 Monotherapy in Japanese patients with Advanced Malignant Solid Tumors	27 (expected)	• Patients with malignant solid tumor • Non-Randomized, Open-label, Sequential assignment • Phase 1 : SAR408701 monotherapy escalating doses/ 4 weeks	• Primary: DLTs, Phase 1 and 1B • Secondary: Safety, Immunogenicity, PK, Plasma CEACAM5 levels, Anti-tumor response RECIST	• SSD: Oct. 2017 • DE: 2019

SAR439859 (SERD)
Breast cancer

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
TED14856 NCT03284957	Phase 1 Phase 2 SAR439859 single agent and in combination with palbociclib in Postmenauposal Women with Estrogen Receptor Positive Advanced Breast Cancer	156	• Non-Randomized, Open-label, Parallel Assignment • Part A: SAR439859 monotherapy dose escalation, • Part C: dose escalation for the combination SAR439859 and palbociclib, • Part B: SAR439859 dose expansion from the dose determined in part A, • Part D: combination SAR439859 and palbociclib at the doses recommended from part C • SAR439859 administered in 28-day cycle; palbociclib in 21-day cycle	• Primary: Parts A & C:DLTs, Parts B & D: ORR • Secondary: Safety, ORR, DCR, DR, PK for both drugs, CYP450 3A induction/inhibition, ER occupancy/PET imaging	• SSD: Sept. 2017 • DE: 2020

SAR440234 (T-cell engaging bispecific Ab) Leukemia

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
TED15138 NCT03594955	Phase 1 Phase 2 SAR440234 single agent in patients with Relapsed or Refractory Acute Myeloid Leukemia (RR AML), B-cell Acute Lymphoblastic Leukemia (B-ALL) or High Risk Myelodysplasia (HR-MDS)	67	• Patients with confirmed diagnosis of AML (except acute promyelocytic leukemia) or MDS with a risk category intermediate or higher, and not eligible for any Tx known to provide clinical benefit, • Open-label, Single Group Assignment • 2 dose escalation schemes, • Cycle defined as 6 weeks of study Tx • Tx may be continued as long as it is clinically beneficial	• Primary: DLTs, allergic reactions/hypersensitivity, ORR, DOR, event-free survival • Secondary: AEs, PK, Preliminary Anti-Leukemia Activity, immunogenicity	• SSD: Nov. 2018 • DE: 2021

SAR442720 (SHP2 inhibitor)
Relapsed/Refractory Solid Tumors

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
RMC-4630-01 NCT03634982	Phase 1 Safety, Tolerability, PK and PD profiles of SAR442720 single agent in patients with Relapsed/Refractory Solid Tumors	200	• Patients with advanced solid tumors that have failed, are intolerant or are considered ineligible for standard of care anticancer Tx • Open-label, Single Group Assignment • 1 Arm: SAR442720, oral administration	• Primary: AEs, DLTs, • Secondary: PK, pERK (PD markers), ORR, DOR,	• SSD: Oct. 2018 • DE: 2021

GZ402666 (avalglucosidase alfa)
Pompe disease (PD) 1/3

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
COMET **Late Onset** EFC14028 NCT02782741	Phase 3 To compare efficacy and safety of Enzyme Replacement therapies avalglucosidase alfa (neoGAA) and alglucosidase alfa (Myozyme®/Lumizyme®) in Tx-naïve patients with Late-onset PD	96	• Repeated Biweekly Infusions of avalglucosidase alfa (GZ402666) and alglucosidase alfa in Tx-naïve patients with late-onset PD age 3 years and older • Randomized, Double-Blind, Parallel Assignment • Total study duration for one patient: 3 years [14-day screening, 49-week blinded Tx period, 96-week open-label Tx and 4-week post-Tx observation period	• Primary: Change in percent predicted forced vital capacity (%FVC) in the upright position, from baseline to 12 months • Secondary: Change from baseline to 12 months in six-minute walk test distance walked, maximal inspiratory / expiratory pressure (% predicted), hand-held dynamometry measurement of lower extremity muscle strength in Quick Motor Function Test scores, and 12- Item Short-form health survey scores	• SSD: Oct. 2016 • DE: 2020

GZ402666 (avalglucosidase alfa)
Pompe disease (PD) 2/3

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
Mini-COMET Infantile Onset ACT14132 NCT03019406	Phase 2 To assess safety and efficacy of avalglucosidase alfa (neoGAA) in Pediatric patients with infantile-onset PD previously treated With alglucosidase alfa (Myozyme®/Lumizyme®)	20	• Repeated bi-weekly infusions of avalglucosidase alfa In Patients with Infantile-onset PD previously treated with alglucosidase alfa (Myozyme®/Lumizyme®) who demonstrate clinical decline or sub-optimal clinical response • Randomized, Open-label, Ascending dose, Parallel assignment • Total study duration for one patient: 3 years [14-day screening, 25-week Tx period, a 120-week extension period and 4-week post-Tx observation period	• Primary: N of participants with AE, N of participants with immunogenicity response Secondary: PK parameters, Change at 6 months from baseline in Gross Motor Function (GMF) Measure-88 Test, revised GMF Classification System score, Pompe specific Pediatric Evaluation of Disability Inventory, Functional Skills Scale, Mobility Domain Test score and Quick Motor Function Test scores, Left Ventricular Mass Index, Eyelid position measurements, Creatine kinase value	• SSD: Oct. 2017 • DE : 2019

GZ402666 (avalglucosidase alfa)
Pompe disease (PD) 3/3

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
NEO-EXT LTS13769 NCT02032524	Phase 2 Phase 3 Long-term safety and PK of repeated biweekly infusions of avalglucosidase alfa (neoGAA) in patients with PD	21	• Repeated biweekly infusions of avalglucosidase alfa In patients with PD who previously completed a avalglucosidase alfa study [adult, senior] • Non-randomized, Open-label, single group assignment • Total study duration for one patient: 6 years [until the patient withdraws, the Investigator withdraws the patient, or the Sponsor terminates the study]	• Primary: AEs and TEAEs, including IARs & deaths, Hematology, biochemistry and urinalysis, vital signs • Secondary: ECG, PK parameters, anti-avalglucosidase alfa antibodies, and neutralizing antibody formation in anti-avalglucosidase alfa positive patients, anti-alglucosidase alfa IgG antibodies, Skeletal muscle glycogen content, Qualitative and quantitative muscle degenerative assessments MRI, Urinary Hex4, plasma analyses of circulating mRNA and micro RNA, Serum analyses of skeletal muscle RNA expression	• SSD: Feb. 2014 • DE: 2020

Olipudase Alfa (rhASM ERT)
Acid Sphingomyelinase Deficiency (ASMD) (1/3)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
ASCEND **Niemann-Pick disease type B**[1] DFI12712 NCT02004691	Phase 2 Phase 3 Efficacy, Safety, PD, and PK study of olipudase alfa in patients with ASMD	36	• Randomized, Double-blinded, Placebo-controlled, Parallel assignment • Study duration is composed of blinded period and an open label extension allowing patients that were on placebo to cross over to active treatment	• Primary: % change in spleen volume, % change in diffusing capacity of the lung for carbon monoxide (Dlco) • Secondary: Change in splenomegaly-related symptom score (except US, where it is part of the primary "combination spleen endpoint"), % change in liver volume, % change in platelet count, **c**hange in fatigue severity as measured by item 3 of the Brief Fatigue Inventory scale, **c**hange in pain severity as measured by item 3 of the Brief Pain Inventory scale, **c**hange in dyspnea severity as measured by the functional assessment of chronic illness therapy dyspnea tool	• SSD: June 2016 • DE (1st Part)[2]: 2019

(1) Non-neurological manifestations of ASMD
(2) Primary analysis period

Olipudase Alfa (rhASM ERT)
Acid Sphingomyelinase Deficiency (ASMD) (2/3)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
ASCEND Peds DFI13803 NCT02292654	Phase 1 Phase 2 Safety, Tolerability, PK, and efficacy evaluation of olipudase alfa in pediatric patients <18 years of age with ASMD	20	• Open-label, ascending dose, Single group assignment • Total study duration for one patient approximately 18 months [up to 60-day screening, 64-week Tx period, 37-day post Tx period except if patient enrolled in a long-term extension study]	• Primary: Safety parameters and Clinically significant changes in laboratory parameters, Clinically significant changes in physical examinations • Secondary: PK parameters, Change in sphingomyelin levels and sphingomyelin metabolite levels	• SSD: June 2015 • DE: 2020

Olipudase Alfa (rhASM ERT)
Acid Sphingomyelinase Deficiency (ASMD) (3/3)

Study	Description	Patients	Design	Endpoints	Status
Long-Term LTS13632 NCT02004704	Phase 2 Long-term study of olipudase alfa in patients with ASDM	25	• For patients who have completed a previous study with olipudase alfa (DFI13803 for pediatric patients, and DFI13412 for adult patients) • Open-label, Single group assignment • Total study duration for one patient: up to 9 years or until marketing approval	• Primary: Safety parameters and physical examinations including neurologic examinations, clinical laboratory tests, inflammatory biomarkers, immune response assessment, vital signs, echocardiogram and electrocardiogram, liver biopsy and liver ultrasound/doppler for patients previously enrolled in DFI13412. • Secondary: Spleen and liver volumes, pulmonary imaging and function tests, hematology and lipid profiles, health outcomes questionnaires For pediatrics patients: Hand X-ray for bone age and bone maturation, Tanner staging and linear patient growth by height Z-score	• SSD: Dec. 2013 • DE(1): 2019

(1) Cut-off to support submission

Venglustat (GCS inhibitor)
Gaucher disease (GD) Type 3

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
LEAP GD Type 3 PDY13949 NCT02843035	Phase 2 Tolerability, PK, PD, and exploratory efficacy of venglustat in combination with cerezyme in adult patients with GD Type 3	10	• 156-week Three part, Open-label, Single group Assignment • Part 1: Evaluate CNS biomarkers in adult GD type 3 patients that distinguish GD3 from GD type 1, Screen adult GD3 patients who qualify for Ttmt with venglustat in Part 2, Total duration 45 days • Part 2 and 3: Safety and tolerability in GD3 patients, Total duration up to 156 weeks including : treatment of 52 weeks (Part 2) and 104 weeks (Part 3) for long term follow-up, respectively	• Primary: N of patients with AE, assessment of PD parameters (GL-1 and lyso GL1) in CSF and plasma • Secondary: PK parameters (CSF and Plasma)	• SSD: Jan. 2017 • DE (1st Part)(1): 2021

(1) Final Data Collection date for primary outcome measure

Venglustat (GCS inhibitor)
Autosomal Dominant Polycystic Kidney Disease (ADPKD)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
STAGED-PKD EFC15392 NCT03523728	Phase 3 Efficacy, safety, tolerability and PK of venglustat in patients at risk of rapidly progressive ADPKD	560	• Randomized, double-blind, placebo-controlled 2-stage study (18 and 24 months) • Study duration per participant is 26 months (maximal) per stage, including a screening period of 15 days, run-in period of 2 weeks, a 24-month treatment period, and a follow-up 30 days after final dose	• Primary: Rate of change in total kidney volume (TKV) based on magnetic resonance imaging (MRI) and rate of change in glomerular filtration rate (eGFR) • Secondary: Rate of change in eGFR, rate of change in TKV, rate of change in urine osmolaity,rate of change in nocturia, adverse events, assessment of PK, change in lens clarity	• SSD: Feb. 2019 • DE (1st Part)(1): 2021

(1) First results available

Venglustat (GCS inhibitor)
Pharmacokinetics in Renal Impairment

Study	Description	Patients	Design	Endpoints	Status
POP14499 NCT03687554	Phase 1 Single dose pharmacokinetic and tolerability study of venglustat in subjects with mild, moderate and severe renal impairment,	24	• Single oral dose under fasting conditions • Single center, open label • For all subjects: male and/or female subjects between 18 and 79 years of age • Subject inclusion conditions: subjects with mild, moderate and severe renal impairment • Duration: Approximately 41 days, including a 21-day screening period, a 1-day treatment period, followed by a 9-day period of plasma sampling for assessment of primary endpoints	• Primary: PK parameters: area under the curve (AUC) Day 1 to 10 • Secondary: PK parameters (Day 1): Cmax. PK parameters (Days 1-10): AUClast, unbound Cmax, unbound AUC, total body clearance from plasma (CL/F) distribution of Venglustat at steady state (Vss/F), fraction of unbound venglustat in plasma (fu), terminal half-life associated with the terminal slope (t1/2z), and effective half-life (t1/2eff). Urine PK parameters (Days 1- 2): cumulated amount Ae0-24, fraction of dose excreted in urine fe0-24, renal clearance (CLR0-24), and predicted accumulation ratio (Rac,pred)	• SSD: Oct. 2018 • DE: 2019

Eliglustat
Gaucher's Disease (GD) (1/2)

Study	Description	Patients	Design	Endpoints	Status
ELIKIDS **GD Type 1/ Type 3** EFC13738 NCT03485677	Phase 3 PK, efficacy and safety with or without Imiglucerase in pediatric patients with GD Type1/Type 3	60	• Non-randomized, open label, two cohort (with and without imiglucerase) • Cohort 1: eliglustat monotherapy • Cohort 2: eligustat plus imiglucerase	• Primary: PK (Cmax and AUC), adverse events • Secondary: changes from baseline as absolute change in g/dL for hemoglobin, % change for platelets, liver volume, and spleen volume; improvement in pulmonary disease, improvement in bone disease, thrombocytopenia, and quality of life	• SSD: Apr. 2018 • DE (1st Part)(1): 2022

(1) Final Data Collection date for primary outcome measure

Eliglustat
Gaucher's Disease (GD) (2/2)

Study	Description	Patients	Design	Endpoints	Status
EXOSKEL **GD Type 1** EFC13781 NCT02536755	Phase 3 Long Term skeletal response to eliglustat in GD Type 1 adult patients who successfully completed Phase 2 or phase 3 studies	32	• Single group assignment, open label	• Primary: change from baseline in bone marrow infiltration, bone mineral density (hips and lumbar spine), skeletal imaging GD bone disease manifestations (lytic lesions, osteonecrosis, fractures and infarcts), clinical GD manifestations (mobility, bone pan, bone crisis), and bone biomarkers • Secondary: quality of life, measurement of GD Type 1 biomarkers and safety (i.e. incidence of adverse events, change from baseline in laboratory assessments (hematology), physical examinations)	• SSD: Oct. 2015 • DE (1st Part)[(1)]: 2019

(1) Final Data Collection date for 2 yr primary outcome measure

SAR339375 (Anti-miR21 RNA)
Alport syndrome (ALPS)(1/2)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
RG012-06 NCT03373786	Phase 1 Safety, efficacy, PD and PK of RG-012 in males and females with ALPS	4	• 18-65 year old males and females with ALPS • Non-randomized, open-label study • Duration: two parts (Part A and Part B). During Part A, half of the participants will receive a single dose and half will receive 4 doses (one dose every other week for 6 weeks). All subjects will undergo two renal biopsies, one before and one after dosing, to assess the effects. After completing Part A, subjects will be able to enter Part B of the study. During Part B, all subjects will receive treatment every other week for 48 weeks	• Primary: Safety (adverse events), and effect on renal microRNA-21 (miR-21) assessed by changes in miR-21 expression in renal tissue • Secondary: PK (Cmax, Tmax and AUC)	• SSD: Dec. 2017 • DE 1st Part)(1): 2019

Information to be reviewed, protocol amendment in preparation

(1) Final Data Collection date for primary outcome measure

SAR339375 (Anti-miR21 RNA)
Alport syndrome (ALPS) (2/2)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
HERA ACT 16248 / RG012-03 NCT02855268	Phase 2a Safety, efficacy, PD and PK of RG-012 in males with ALPS	40	• 18-60 year old males with ALPS • Randomized, double-blind, placebo-control, multi-center • Duration: 48 week SC injections double-blinded treatment period. After 48 week treatment, subjects can receive a 48 week open-label extension period	• Primary: Safety and tolerability assessed by frequency and severity of AEs, changes in laboratory parameters, vital signs and ECGs	• SSD: Nov. 2017 • DE: 2019

Study suspended, Information to be reviewed, protocol amendment in preparation

Teriflunomide
Multiple Sclerosis (MS)

Study	Description	Patients	Design	Endpoints	Status
TERIKIDS RMS EFC11759 NCT02201108	Phase 3 Efficacy, Safety and PK of teriflunomide in Pediatric Patients With Relapsing Forms of MS	165	• Patients with RMS meeting the criteria of MS based on McDonald criteria 2010 and International Pediatric MS Study Group criteria for pediatric MS • With at least one relapse (or attack) in the 12 months preceding randomization or at least two relapses (or attack) in the 24 months preceding randomization • Randomized, Double-Blind, Placebo-Controlled, Parallel Group , Tx 96 weeks followed by Open-label extension (96 weeks up to a max of 192 weeks after randomization), follow-up 4 weeks after Tx discontinuation	• Primary: Time to first clinical relapse after randomization • Secondary: % of relapse free patients, N of new/newly enlarged T2 lesions, N of T1 Gd-enhancing T1 lesions , Change in volume of T2 lesions , of T1 hypointense lesions , brain atrophy, % of patients free of new or enlarged MRI T2-lesions, Change in performance on SDMT and Cognitive Battery Test , Safety, PK	• SSD: Jul. 2014 • DE(1st Part)(1): 2019

(1) Final Data Collection date for primary outcome measure

Alemtuzumab
Relapsing Remitting Multiple Sclerosis (RRMS)

Study	Description	Patients	Design	Endpoints	Status
LemKids RRMS EFC13429 NCT03368664	Phase 3 Efficacy, Safety and Tolerability of Alemtuzumab in Pediatric Patients With Relapsing Remitting MS (RRMS) with disease activity on prior disease modifying therapy DMT	50	• In pediatric patients from 10 to <18 years of age with RRMS with disease activity on prior DMT. • Open-label, rater-blinded, single-arm, cross-over study The study will consist of different phases: • Prior DMT Phase (~4 months) – efficacy measurements on current DMT • Alemtuzumab Treatment Phase (~2 years) - The MRI based primary efficacy endpoint will be assessed over a 4 month period during this phase compared to an equal period during the prior DMT phase • Safety Monitoring Phase – safety monitoring for all patients treated with alemtuzumab (4 years post last treatment with alemtuzumab)	• Primary: The number of new or enlarging T2 lesions on brain MRI, during continuation of prior DMT (Period 1) compared to an equal period after the first course of alemtuzumab treatment (Period 2) • Secondary: The proportion of patients with new or enlarging T2 lesions , Annualized relapse rate at Year 2, Assessment of cognition test scores, Additional secondary endpoints, including PK/PD parameters and Quality of Life (QoL) measures.	• SSD: Oct. 2017 • DE: 2025

SAR422459 (ABCA4 gene therapy)*
Stargardt Disease

Study	Description	Patients	Design	Endpoints	Status
Stargardt's Macular Degeneration TDU13583 NCT01367444	Phase 1 Phase 2/2a Safety and tolerability of ascending doses of SAR422459 in patients with Stargardt's Macular Degeneration	46	• Patients with a diagnosis of Stargardt's Macular Degeneration, with at least one pathogenic mutant ABCA4 allele on each chromosome • Non-randomized, Single Group assignment, Open-label, ascending doses	• Primary: IAE, Change from baseline in ocular safety assessments • Secondary: Delay in retinal degeneration	• SSD: Jun. 2011 • DE: 2020
Stargardt's Macular Degeneration LTS13588 SG1/002/11 NCT01736592	Phase 1/2 Follow-up study of SAR422459 in patients With Stargardt 's Macular Degeneration	46	• Long Term safety and tolerability of SAR422459 in patients with Stargardt 's Macular Degeneration • No ttmt administered, in this LTS only follow-up after ttmt in TDU13583 • Patients will be followed for 15 years after treatment	• Primary: IAE • Secondary: Delay in retinal degeneration	• SSD: Dec. 2012 • DE: 2034

* Identification of out-licensing partner ongoing

SAR421869 (Myosin 7A gene therapy)*
Usher 1B Syndrome

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
UshStat® **Usher Syndrome Type 1B** TDU13600 NCT01505062	Phase 1 Phase 2a Safety and tolerability of ascending doses of subretinal injections of UshStat® in patients with Retinitis Pigmentosa associated with Usher syndrome Type 1B	18	• Patients with clinical and molecular diagnosis of Retinitis Pigmentosa associated with Usher Syndrome type 1B. With at least one pathogenic mutation in the MYO7A gene on each chromosome • Non-randomized, Single Group assignment, Open-label, ascending doses	• Primary: IAE • Secondary: Delay in retinal degeneration	• SSD: Mar. 2012 • DE: 2021
UshStat® **Usher Syndrome Type 1B** LTS13619 NCT02065011	Phase 2b Long-Term Safety, Tolerability and Biological Activity of UshStat® in Patients With Usher Syndrome Type 1B	18	• Long-term follow up of patients who received UshStat® in a previous study (TDU13600) • Patients will be followed for 15 years after treatment	• Primary: IAE • Secondary: Change from baseline in ocular safety assessments, Delay in retinal degeneration	• SSD: Sep. 2013 • DE: 2036

* Project discontinued and identification of out-licensing partner ongoing

Venglustat (GCS inhibitor)
GBA-PD

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
MOVES-PD ACT14820 NCT02906020	Phase 2 Efficacy, safety, pharmacokinetics, and pharmacodynamics of venglustat (GZ402671) in patients with Parkinson's Disease (PD) carrying a glucocerebrosidase gene (GBA) mutation	243	• Patients with PD carrying a GBA mutation or other prespecified variant. • Randomized, Double-blind, Placebo Controlled, Parallel Assignment • Part 1: Increasing dose of venglustat administered once per day. Duration: up to 48 weeks outside Japan, and up to 64 weeks in Japan • Part 2: venglustat dose determined in Part 1 administered once a day Duration: 5,6-week screening, 52-week Tx period, 104-week follow-up period and 6-week post Tx observation	• Primary: Change from baseline in Movement Disorder Society Unified PD Rating Scale Part II and III score • Secondary: Change from baseline in PD Cognitive Rating Scale, Movement Disorder Society Unified PD Rating Scale Part I, II, and III score, Hoehn and Yahr score	• SSD: Dec. 2016 • DE 1st Part)(1): 2021

(1) Final Data Collection date for primary outcome measure

SAR443060 (DNL747) (RIPK1 inhibitor)
Alzheimer's Disease

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
DNLI-D-0002 NCT03757325	Phase 1* Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of SAR443060(DNL747) in Subjects with Alzheimer's disease	16	• Patients suffering from Alzheimer's Disease • Randomized, Double-blind, Placebo Controlled, Cross-over Assignment • SAR443060 and placebo	• Primary: AEs and SAEs, lab test abnormalities • Secondary: Pharmacokinetics, Pharmacodynamics	• SSD: Dec. 2018 • DE: 2019

* Phase 1 study performed by Denali

SAR443060 (DNL747) (RIPK1 inhibitor)
Amyotrophic Lateral Sclerosis (ALS)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
DNLI-D-0003 NCT03757351	Phase 1* Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of SAR443060(DNL747) in Subjects with Amyotrophic Lateral Sclerosis	16	• Patients with a diagnosis of laboratory-supported probable, probable or definite ALS • Randomized, Double-blind, Placebo Controlled, Cross-over Assignment • SAR443060 and placebo	• Primary: AEs and SAEs, lab test abnormalities, clinically significant neurological abnormalities • Secondary: Pharmacokinetics, Pharmacodynamics	• SSD: Dec. 2018 • DE: 2019

* Phase 1 study performed by Denali

Lixisenatide
Type 2 Diabetes Mellitus (T2DM) Pediatrics

Study	Description	Patients	Design	Endpoints	Status
TDR14311 NCT02803918	Phase 1 PK and PD of lixisenatide in Pediatric Patients With T2DM not adequately controlled with metformin and/or basal insulin	24	• Pediatric patients (≥ 10 and < 18 years old with documented T2DM insufficiently controlled with metformin and/or basal insulin • Randomized, double-blind, placebo-controlled, dose escalation (3 ascending repeated doses) • Study duration: up to 10 weeks including 6-week Tx period with dose escalation every 2 weeks	• Primary: AEs, TEAEs, number of patients with anti-lixisenatide Ab, • Secondary: lixisenatide PK parameters, PD (plasma glucose AUC-0-4,5 hours)	• SSD: May 2017 • DE: 2020

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-MONO (301) **T2DM** **EFC14833** NCT02926937	Phase 3 Efficacy and safety of sotagliflozin vs. placebo in patients with T2DM not currently treated with antidiabetic therapy	400	• Patients (male and female) with T2D, who are treated with diet and exercise only during the 12 weeks prior to screening • Randomized, Double-blind, Placebo-controlled, Parallel-group, 3-Tx arm, sota dose 1/200mg, sota dose 2/400mg, placebo • Study duration: up to 34-week: up to 2-week screening period, 2-week single-blind placebo run-in, 26-week double-blind Tx period and 4-week post Tx follow-up	• Primary: Change from Baseline in HbA1c in comparison of sotagliflozin dose 1 vs. placebo • Secondary: Change from baseline in 2-hour PPG following a mixed meal in comparison of sotagliflozin doses 1/2 vs. placebo, FPG in comparison of sotagliflozin dose 1 vs. placebo, Body weight in comparison of sotagliflozin doses 1/2 versus placebo, % of patients with HbA1c <6.5% in comparison of sotagliflozin dose 1 vs. placebo, % of patients with HbA1c <7.0% in comparison of sotagliflozin dose 1 vs. placebo, Change from Baseline in HbA1c in comparison of sotagliflozin dose 2 vs. placebo, Change from baseline in SBP for patients with baseline SBP ≥130 mmHg in comparison of sotagliflozin dose 1 vs. placebo and SBP for all patients in comparison of sotagliflozin doses 1/2 vs. placebo	• SSD: Nov 2016 • DE: 2019

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-MET (302) **T2DM** **EFC14834** NCT02926950	Phase 3 Efficacy and safety of sotagliflozin added to metformin in patients with T2DM who have inadequate glycemic control on metformin	500	• Patients with T2DM currently treated with diet and exercise and on metformin at a stable dose ≥1500 mg/day for at least 12 weeks • Randomized, Double-blind, Placebo-controlled, Parallel-group, 2-Tx arm (placebo – sota 400mg), On top of metformin • Study duration: up to 87-week: up to 2-week screening period, 2-week single-blind placebo run-in, 26-week double-blind core Tx period , 53-week double-blind extension period and 4-week post Tx follow-up	• Primary: Change from Baseline in HbA1c • Secondary: Change from Baseline I in 2-hour PPG following a mixed meal, in FPG, in body weight % of patients with HbA1c <6.5% - % patients with HbA1c <7.0% Change from Baseline I in systolic blood pressure (SBP) for patients with baseline SBP ≥130 mmHg in SBP for all patients.	• SSD: Nov 2016 • DE: 2019

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-SU (307) T2DM **EFC14835** NCT03066830	Phase 3 Efficacy and safety of sotagliflozin added to a sulfonylurea alone or in combination with metformin in patients with Type 2 Diabetes who have inadequate glycemic control on a sulfonylurea alone or with metformin	500	• Patients with T2DM treated with a sulfonylurea (≥half the maximum recommended dose as per local label or MTD as monotherapy or in combination with metformin (≥1500 mg per day or MTD) each at a stable dose for at least 12 weeks • Randomized, Double-blind, Placebo-controlled, Parallel-group, 2-Tx arm (placebo – sota 400mg) • On top of sulfonylurea alone or in combination with metformin • Study duration: up to 85-week: up to 2-week screening period, 2-week single-blind run-in, 26-week double-blind core Tx period, 53-week double-blind extension period and 2-week post Tx follow-up	• Primary: Change from Baseline in HbA1c • Secondary: Change from baseline in FPG, in body weight, in Systolic Blood Pressure (SBP) for patients with baseline SBP ≥130 mmHg, in SBP for all patients, % of patients with HbA1c <6.5%, % of patients with HbA1c <7.0%	• SSD: Feb 2017 • DE: 2019

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-CKD3 (306) **T2DM** **EFC14837** NCT03242252	Phase 3 Evaluate the efficacy and safety of sotagliflozin in patients with T2DM and Moderate Renal Impairment who have inadequate glycemic control	780	• Patients with T2DM (drug-naïve or on antidiabetic therapy) and documented moderate renal insufficiency defined by an estimated glomerular filtration rate (based on the 4 variable Modification of Diet in Renal Disease equation) of ≥30 and <60 mL/min/1.73 m2 (CKD 3A, 3B) • Randomized, Double-blind, Placebo-controlled, Parallel-group, 3-Tx arm (placebo – sota 200mg - sota 400mg) • Study duration: up to 60-week: up to 2-week screening period, 2-week single-blind run-in, 52-week randomized Tx period and 4-week post Tx follow-up	• Primary: Change in HbA1c for sota dose 1 and sota dose 2 • Secondary: Change from Baseline in FPG (doses 1/2) in SBP for patients with baseline SBP ≥130 mmHg (doses 1/2), in SBP for all patients (doses 1/2) and in body weight (doses 1/2), % change in UACR for patients with UACR > 30 mg/g (doses 1/2), % of patients with HbA1c less than 6.5% (doses 1/2), % of patients with HbA1c less than 7.0% (doses 1/2), % of patients with AE (doses 1/2)	• SSD: Aug 2017 • DE: 2019

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-CKD4 (306) **T2DM** **EFC15166** NCT03242018	Phase 3 Evaluate the efficacy and safety of sotagliflozin in patients with T2DM and severe renal impairment who have inadequate glycemic control	276	• Patients with T2DM (drug-naïve or on antidiabetic therapy) and documented severe renal insufficiency - CKD4 - defined by an estimated glomerular filtration rate equation (based on the 4 variable modification of diet in renal disease equation) of ≥15 and <30 mL/min/1.73 m2 • Randomized, Double-blind, Placebo-controlled, Parallel-group, 3-Tx arm (placebo – sota 200mg - sota 400mg) • Study duration: up to 60-week: up to 2-week screening period, 2-week single-blind run-in, 52-week randomized Tx period and 4-week post Tx follow-up	• Primary: Change from Baseline in HbA1c comparing sotagliflozin dose 1 vs. placebo in CKD4 patients • Secondary: Change from baseline in HbA1c comparing sotagliflozin dose 2 vs. placebo, in FPG (doses 1/2), in SBP at for patients with SBP greater than or equal to 130 mmHg (doses 1/2), in SBP in all patients (doses 1/2), in body weight (doses 1/2), % change in the UACR for patients with a UACR > 30 mg/g at baseline (doses 1/2), % of patients with HbA1c less than 6.5% (doses 1 and 2), % of patients with HbA1c less than 7.0% (doses 1 and 2), N of patients with AE (doses 1/2)	• SSD: Aug 2017 • DE: 2019

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-INS (312) T2DM **EFC14868** NCT03285594	Phase 3 Efficacy and safety of sotagliflozin in patients with T2DM who have inadequate glycemic control on Basal Insulin alone or in addition to Oral Antidiabetes Drugs (OADs)	560	• Patients with T2DM using any types of basal insulin alone or in combination with up to 2 OADs • Randomized, Double-blind, Placebo-controlled, Parallel-group, 3-Tx arm (placebo – sota 200mg - sota 400mg) • Background therapy with insulin glargine (Lantus®) (with or without OADs) throughout the study • Study duration: up to 60-week: up to 2-week screening period, 4-week Lantus® titration single-blind placebo run-in period, 52-week double-blind Tx period and 2-week post Tx follow-up	• Primary: Absolute change in hemoglobin A1c (HbA1c) (for sotagliflozin dose 1) • Secondary: Change in FPG (for sotagliflozin doses 1/2), in Body Weight (for sotagliflozin doses 1/2), in HbA1c (for sotagliflozin dose 1/2), in SBP for patients with baseline SBP ≥130 mmHg (for sotagliflozin doses 1/2), in SBP for all patients (for sotagliflozin dose 1),	• SDD: Sep 2017 • DE: 2019

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SCORED (303) T2DM **EFC14875** NCT03315143	Phase 3 Effects of sotagliflozin on CV and renal events in patients with T2DM, CV risk factors and moderately impaired renal function	10 500	• Patients : T2DM with glycosylated hemoglobin (HbA1c) ≥ 7%, Estimated glomerular filtration rate (eGFR) ≥ 25 and ≤ 60 mL/min/1.73 m2, Age 18 years or older with at least one major CV risk factor or age 55 years or older with at least two minor CV risk factors • Randomized, Double-blind, Placebo-controlled, Parallel-group, 2-Tx arm (placebo - sota 400mg) • Total Study duration: approximately 27 to 51 months, 24-month recruitment and 27-month of follow-up after the last patient randomized	• Primary: Baseline to approx. 51 months, Time to the first occurrence of any of the following clinical events: CV death, Non-fatal myocardial infarction, Non-fatal stroke, Time to the first occurrence of any of the following clinical events: CV death; Hospitalization for heart failure • Secondary: Baseline to approx. 51 months, Time to first composite renal event, Time to first composite renal event in subgroup of patients with macroalbuminuria, Total Number of heart failure events, time to CV death , time to all cause mortality	• SSD: Nov. 2017 • DE: 2022

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
GLIM (304) T2DM **EFC14838** NCT03332771	Phase 3 Efficacy and safety of sotagliflozin vs. glimepiride and placebo in patients with T2DM that are taking metformin monotherapy	930	• Patients : Patients with T2DM treated with metformin at a stable dose ≥1500 mg/day or MTD (documented) for at least 12 weeks prior to screening visit • Randomized, Double-blind, Double-dummy, Active and Placebo-controlled, Parallel-group, 4-Tx arm (placebo – glimepiride, sota dose 1, sota dose 2) • Total Study duration: up to 58 weeks including 2-week screening phase, 2-week single-blind placebo run-in, 52-week double-blind Tx period and 2-week post Tx follow-up	• Primary: Absolute change in hemoglobin A1c (HbA1c) (for sotagliflozin dose 1) • Secondary: Change in Body Weight (for sotagliflozin dose 1), in HbA1c (for sotagliflozin dose 2), in SBP for patients with baseline SBP ≥130 mmHg (for sotagliflozin dose 1), in SBP for all patients (for sotagliflozin dose 1), % of patients with at least one hypoglycemic event (for sotagliflozin dose 1), % of patients with AE	• SSD: Dec 2017 • DE: 2019

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
T2DM & Mild to Moderate HTA **PDY15010** NCT03462069	Phase 2 Multiple Dose Study to Assess the Intestinal, Metabolic and Cardiovascular Effects of an 8 Weeks Treatment With Sotagliflozin Once a Day (QD) as Compared With Empagliflozin QD in Type 2 Diabetes Mellitus (T2DM) Patients With Mild to Moderate Hypertension	40	• T2 DM patients with Hypertension grades 1 or 2 diagnosed for at least 1 year • A Randomized, Double-blind, Parallel-group, 2-treatment Multiple Dose Study • Two arms: Treatment A (test): Sotagliflozin 2 tablets administered once daily with 1 empagliflozin placebo capsule prior to the first meal of the day Treatment B (Reference) Empagliflozin 1 capsule administered once daily with 2 sotagliflozin placebo tablets prior to the first meal of the day	• Primary: PD parameters in feces: (sodium excretion, SCFA, pH), urine: glucose & sodium excretion, blood: 14 h plasma glucose profile and GLP-1 profile after standardized meals • Secondary: change in fasting plasma glucose, ABPM, change in plasma aldosterone, change in carotid-femoral pulse wave velocity, CGM, LVEF, change in left ventricular end-diastolic diameter, change in plasma volume, AEs, PK	• SSD: Mar. 2018 • DE: 2019

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-BONE **EFC15294** NCT03386344	Phase 3 Efficacy and Bone safety of sotagliflozin in Patients 55 years or older with T2DM and Inadequate Glycemic Control	360	• Patients with T2DM managed with diet and exercise only or with a stable antidiabetes regimen for more than 12 weeks, 55 years or older • A Randomized, Double-blind, Parallel-group, • Three arms: Treatment Sotagliflozin (dose 1 and dose 2), placebo 26-week Tx, with 78-week double blind extension period	• Primary: HbA1c, change (dose 1) • Secondary: Bone mineral density (BMD) of lumbar spine, total hip, and femoral neck, change (dose 1 and 2), Hb1Ac change (dose 2), BW, FPG, SBP, and % of patients with Hb1Ac < 7%, change (dose 1 and 2), AEs	• SSD: Feb 2018 • DE: 2020

Sotagliflozin (SGLT-1&2 inhibitor)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-EMPA **EFC14867** NCT03351478	Phase 3 Efficacy and Safety of sotagliflozin vs placebo and empagliflozin in Patients with T2DM who have inadequate glycemic control while taking DPP4 inhibitor alone or with metformin	700	• Patients with T2DM on DPP4 with or without metformin at a stable dose for at least 12 weeks prior to screening • A Randomized, Double-blind, Parallel-group, • Three arms: Sotagliflozin, empagliflozin, placebo • Study duration: up to 34 weeks, including a screening phase up to 2 weeks, a 2-week run-in phase, a 26-week double-blind Tx period, and a 4-week Tx FU	• Primary: HbA1c, change • Secondary: SBP in patients with SBP ≥ 130mmHg, PPG following mixed meal tolerance test (MMTT), FPG, BW, SBP, patients with Hb1Ac < 6,5%, % of patients with Hb1Ac < 7%	• SSD: Nov 2017 • DE: 2019

Sotagliflozin (SGLT-1&2 inhibitor)
Worsening Heart Failure

Study	Description	Patients	Design	Endpoints	Status
Worsening Heart Failure **PDY15079** NCT03292653	Phase 2 Safety, Tolerability and Pharmacodynamic activity of sotagliflozin in Hemodynamically Stable Patients with Worsening Heart Failure	81	• Patients: Admitted to the hospital with worsening of heart failure • Design: Randomized, double-blind, placebo-controlled study consisting of 3 subsequent cohorts. Cohort 1: sotagliflozin 200 mg (n=10) or placebo (n=5) ; Cohort 2: sotagliflozin 400 mg (n=10) or placebo (n=5): Cohort 3: sotagliflozin 200 mg (n=17), 400 mg (n=17) or placebo (n=17) • Treatment duration: In each cohort, study treatment is administered orally for 14 days	• Primary: Safety and Tolerability; Pharmacodynamics: Changes in hemoconcentration from baseline to 14 days, Changes in plasma volume from baseline to 14 days • Secondary: Change in erythropoietin from baseline to 14 days, Change in NT-proBNP from baseline to 14 days	• SSD: Dec. 2017 • DE: 2019

Sotagliflozin (SGLT-1&2 inhibitor)
Worsening Heart Failure

Study	Description	Patients	Design	Endpoints	Status
SOLOIST-WHF **EFC15156** NCT03521934	Phase 3 Effects of sotagliflozin on Clinical Outcomes in Hemodynamically Stable Patients with T2DM POST Worsening Heart Failure	4000	• Patients with T2DM, admitted to the hospital or urgent heart failure visit for worsening heart failure • Design: Randomized, double-blind, placebo-controlled, parallel - group • Two Arms: sotagliflozin, placebo • Estimated study duration for a given patient: approximately 3 to 32 months	• Primary: Time to 1st occurrence of either CV death or hospitalization for heart failure (HHF) in patients with LVEF < 50%, Time to 1st occurrence of either CV death or HHF in the total patient population • Secondary: Total number of CV death, HHF or urgent HF visit (including recurrent events), Time to first occurrence of composite renal endpoint, Time to CV death in patients with LVEF < 50%, Time to CV death in the total patient population, Time to all cause mortality in patients with LVEF < 50%, Time to all cause mortality in the total patient population	• SSD: June 2018 • DE: 2021

Efpeglenatide (Long acting GLP1-R agonist)
Type 2 Diabetes Mellitus

Study	Description	Patients	Design	Endpoints	Status
AMPLITUDE-M **EFC14822** **NCT03353350**	Phase 3 Efficacy and Safety of efpeglenatide in Patients with T2DM Inadequately Controlled with Diet and Exercise	400	• A 56-week, multicenter, double-blind, • placebo-controlled, 4 parallel arms, randomized study to demonstrate the superiority of once-weekly injection of efpeglenatide 2, 4, or 6 mg in comparison to placebo in HbA1c change from baseline to Week 30	Primary: change in HbA1c (%) from Baseline to Week 30 Secondary • Number of participants with HbA1c <7.0% at Week 30 • Change from Baseline to Weeks 30 and 56 in fasting plasma glucose • Change in HbA1c (%) from Baseline to Week 56 • Change from Baseline to Weeks 30 and 56 in body weight • Number of patients with at least one hypoglycemic event during treatment period • Number of hypoglycemic events per participant-year during treament period • Number of patients with AEs	• SSD: Dec. 2017 • DE: 2020

Efpeglenatide (Long acting GLP1-R agonist)
Type 2 Diabetes Mellitus

Study	Description	Patients	Design	Endpoints	Status
AMPLITUDE-O **EFC14828** **NCT03496298**	Phase 3 Effects of efpeglenatide on Cardiovascular outcomes in high cardiovascular risk T2DM patients	4000	• T2DM patients with Hb1Ac > 7% with either established cardiovascular disease or renal impairment 25 ≤ eGFR < 60 mL/min and at least one cardiovascular risk factor • Randomized, double-blind, placebo-controlled, parallel-group (efpeglenatide 4mg, 6mg, placebo) • Estimated study duration per patient up to 36 months approximately • Study is event driven; mean follow up of 2,5 years is expected	• Primary: time to first Major Adverse Cardiovascular Event (MACE) • Secondary: time to first, Expanded Cardiovascular Outcome event, Composite Renal event, AEs	• SSD: Apr. 2018 • DE: 2021

Efpeglenatide (Long acting GLP1-R agonist)
Type 2 Diabetes Mellitus

Study	Description	Patients	Design	Endpoints	Status
AMPLITUDE-D **EFC14829** NCT03684642	Phase 3 Efficacy and safety of efpeglenatide vs duraglutide in patients with T2DM inadequately controlled with metformin	900	• Patients with T2DM on HBA1c between 7-10% (inclusive) on a stable dose of at least 1500 mg metformin or tolerated maximum dose for at least 3 months prior to screening • Randomized, multi-center, open-label for the drug (efpeglenatide and dulaglutide) and double-blind for the doses of efpeglenatide, active-controlled • Three arms: efpeglenatide 4, or 6 mg vs dulaglutide • Study duration: overall 56 weeks	• Primary: change from baseline to week 56 in HbA1c, • Secondary: - Change from baseline to week 56 in FPG - Change from baseline to week 56 in body weight - Number of patients with HbA1c < 7% at week 56 - Number of patients with at least one hypoglycemic event during treatment period - Number of hypoglycemic events per participant-year during treatment period - Number of patients with Aes	• SSD: Oct. 2018 • DE: 2020

Efpeglenatide (Long acting GLP1-R agonist)
Type 2 Diabetes Mellitus

Study	Description	Patients	Design	Endpoints	Status
AMPLITUDE-L **EFC14893** NCT03713684	Phase 3 Efficacy and safety of efpeglenatide vs placebo in patients with T2DM inadequately controlled with basal insulin alone or in combination with oral antidiabetic drug(s)	400	• Patients with T2DM on HBA1c between 7-10% (inclusive) on basal insulin alone or in combination with oral antidiebetic drug(s) at a stable dose for at least 6 months prior to screening • Randomized, multi-center, double-blind, parallel-arms, parallel groups • Four arms: efpeglenatide 2, 4, or 6 mg vs placebo • Study duration: overall 56 weeks	• Primary: change from baseline to week 30 in HbA1c, • Secondary: - Change from baseline to week 56 HBA1c - Change from baseline to week 56 in FPG - Number of patients with HbA1c < 7.0% at week 30 - Change from baseline to week 30 and week 56 in body weight - Number of patients with at least one hypoglycemic event during treatment period - Number of hypoglycemic events per participant-year during treatment period - Number of patients with AEs	• SSD: 2019 • DE: 2021

Efpeglenatide (Long acting GLP1-R agonist)
Type 2 Diabetes Mellitus

Study	Description	Patients	Design	Endpoints	Status
AMPLITUDE-S **EFC15337** NCT03770728	Phase 3 Efficacy and safety of efpeglenatide vs placebo in patients with T2DM inadequately controlled with controlled with metformin alone or in combination with sulfonylurea	640	• Patients with T2DM on HBA1c between 7-10% (inclusive) on metformin with or without sulfunylurea at a stable dose for at least 12 weeks prior to screening • Randomized, multi-center, double-blind, parallel-arms, parallel groups • Four arms: • efpeglenatide 2, 4, or 6 mg, placebo • Study duration: 30 weeks,	• Primary: change from baseline to week 30 in HbA1c • Secondary: - Number of patients with HbA1c < 7.0% at week 30 - Change from baseline to week 56 in FPG - Change from baseline to week 30 in body weight - Number of patients with at least one hypoglycemic event during treatment period - Number of hypoglycemic events per participant-year during treatment period - Number of patients with Aes	• SSD: 2019 • DE: 2021

SAR341402 (Rapid Acting Insulin)
Type 1 & 2 Diabetes Mellitus

Study	Description	Patients	Design	Endpoints	Status
GEMELLI 1 **EFC15081** NCT03211858	Phase 3 Comparison of SAR341402 to NovoLog®/NovoRapid® in adult patients with Diabetes also using Insulin Glargine, with a 6-month safety extension period	597	• Patients with T1DM or T2DM diagnosed for at least 12 months, who have been treated with a multiple daily injection regimen with NovoLog®/NovoRapid® OR insulin lispro (100 U/mL) in the last 6 months prior to screening visit AND insulin glargine (100 U/mL) in the last 6 months prior to screening visit OR insulin detemir (Levemir®) in the last 12 months prior to screening visit • Randomized, Open-label, Parallel-group • Active comparator: NovoLog®/NovoRapid® • Study duration: 54-week per patient: 2-week screening period, 26-week Tx period, 26-week comparative safety extension, 1-day follow-up period	• Primary: Change in HbA1c (%) from baseline to Week 26 • Secondary: Change in HbA1c, Patients with HbA1c <7%, Change in FPG, Change in mean 24-hour plasma glucose concentration, Change in PPG, Change in 7-point SMPG, Hypoglycemic patients, Hypoglycemic events, Anti-SAR341402/NovoLog/NovoRapid antibody status, Tx-induced, Tx-boosted and Tx-emergent anti-insulin antibodies	• SSD: Aug. 2017 • DE: 2019

Alirocumab (anti-PCSK-9 mAb)
Heterozygous Familial Hypercholesterolemia (HeFH) (1/2)

Study	Description	Patients	Design	Endpoints	Status
ODYSSEY KIDS DFI14223 NCT02890992	Phase 2 Efficacy and safety of alirocumab in children and adolescents with heFH followed by an extension phase	42	• Patients with diagnosis of HeFH through genotyping or clinical criteria., 8 to 17 years old, treated with optimal dose of statin +/- other LMT(s) or non-statin LMT(s) if statin intolerant at stable dose for at least 4 weeks prior to screening lipid sampling • Open-Label, Sequential, Repeated Dose-Finding Study (6 doses tested) • Background therapies: optimal dose of statin with or without other LMT or non-statin LMT if statin intolerant at stable dose • Study duration: approximately 16-23 weeks: up to 6 (+1) weeks screening period, 8 weeks open-label Tx period, 6 to 8 weeks follow-up period	• Primary: % change in calculated LDL-C from baseline to week 8 • Secondary: Absolute change in calculated LDL-C, % change in APO B (Apo B), % change in non-high density LP cholesterol (non HDL-C), % change in Total-C, in LP, in TG, in HDL-C, in Apo A-1, Absolute change in Apo B, in non-HDL-C, in Total C, in Lp(a), in TG, in HDL-C, in Apo A-1, in ratio apo B/Apo A-1, % of participants achieving a calculated LDL-C level lower than 130 mg/dL (3.37 mmol/L), % of participants achieving a calculated LDL-C level lower than 110 mg/dL (2.84 mmol/L)	• SSD: Sep. 2016 • DE: 2019

Alirocumab (anti-PCSK-9 mAb)
Heterozygous Familial Hypercholesterolemia (HeFH) (2/2)

Study	Description	Patients	Design	Endpoints	Status
HeFH in Children and Adolescents EFC14643 NCT03510884	Phase 3 Efficacy and safety of alirocumab in children and adolescents with HeFH	150	• Patients with diagnosis of HeFH, 8 to 17 years old, treated with optimal dose of statin +/- other LMT(s) or non-statin LMT(s) if statin intolerant at stable dose • Randomized, double-Blind, placebo-controlled followed by an open label treatment period (2 dose tested) • Study duration: approximately 110 weeks (run-in period, if needed,: up to 4 weeks [+2 days], screening period, up to 2 weeks (+5 days), double-blind treatment period: 24 weeks, open label treatment: 80 weeks)	• Primary: % change in LDL-C from baseline to week 24 • Secondary: % change in LDL-C, % change in APO B (Apo B), % change in non-high density LP cholesterol (non HDL-C), % change in Total-C, patients with LDL-C level lower than 130 mg/dL (3.37 mmol/L), patients with LDL-C level lower than 110 mg/dL (2.84 mmol//L), % change in Lp(a), in HDL-C, in TG and in ApoA-1. Number of AE, maturing cognition (Cogstate battery test) and pubertal development (Tanner stage)	• SSD: May 2018 • DE: 2022

Alirocumab (anti-PCSK-9 mAb)
Homozygous Familial Hypercholesterolemia (HoFH) (1/2)

Study	Description	Patients	Design	Endpoints	Status
ODYSSEY HoFH **Regeneron** R727-CL-1628 NCT03156621	Phase 3 Evaluate the efficacy and safety of alirocumab in patients with HoFH	54	• Diagnosis of HoFH by specific genotype or clinical criteria (all patients on LDL apheresis must be diagnosed based on genotype) • Randomized, Double-Blind, Placebo-Controlled, Parallel-Group, 2-Arm (alirocumab Q2W, placebo) • Study duration: 12-week double-blind Tx period followed by 10-week alirocumab open-label Tx period	• Primary: % change in LDL-C ITT population from baseline to week 12 • Secondary: % change in Apo B, % change in non-HDL-C, % change in TC, % change in LP(a), % change in HDL-C, % change in fasting TG, % change in Apo A-1, % change in LDL-C, % change in LDL-C, ApoB B, non-HDL-C, TC, Lp(a), HDL-C, fasting TG, Apo A-1 / (m)ITT population, Absolute change in the ratio of Apo B/Apo A-1 (*ITT)*, % of patients with ≥15% reduction in LDL-C, % of patients with ≥30% reduction in LDL-C, % of patients with ≥50% reduction in LDL-C, % of patients with ≥15% reduction, ≥30% reduction, and ≥50% reduction in LDL-C	• SSD: Oct. 2017 • DE: 2019

Alirocumab (anti-PCSK-9 mAb)
Homozygous Familial Hypercholesterolemia (HoFH) (2/2)

Immuno-inflammation | Diabetes
Oncology | **Cardiovascular**
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
HoFH Children and Adolescents EFC14660 NCT03510715	Phase 3 Efficacy and safety of alirocumab in children and adolescents with HoFH	18	• Patients with diagnosis of HoFH, 8 to 17 years old, treated with optimal dose of statin +/- other LMT(s) or non-statin LMT(s) if statin intolerant at stable dose • Single group assignment, open label (2 doses) • Study duration: up to 62 weeks, includes (if needed) a run-in period of up to 4 weeks, a screening period of up to 2 weeks, a treatment period of up to 48 weeks, and a follow-up of 8 weeks	• Primary: % change in calculated LDL-C from baseline to week 12 • Secondary: % change in LDL-C, % change in APO B (Apo B), % change in non-high density LP cholesterol (non HDL-C), % change in Total-C, % change in Lp(a), in HDL-C, in TG and in ApoA-1. Absolute change in LDL-C, number of patients with AE and pubertal development (Tanner stage)	• SSD: Sep. 2018 • DE: 2020

Alirocumab (anti-PCSK-9 mAb)
Neurocognitive Evaluation (1/2)

Study	Description	Patients	Design	Endpoints	Status
Neurocognitive Evaluation **Regeneron** R727-CL-1532 NCT02957682	Phase 4 Evaluate the effect of alirocumab on Neurocognitive function in patients with HeFH and non-HeFH at high and very high cardiovascular risk	2176	• Patients with hypercholesterolemia and established coronary heart disease (CHD) or CHD risk equivalents who are not adequately controlled with a maximally tolerated daily dose of statin at a stable dose for at least 4 weeks prior to the screening visit • Randomized, Double-Blind, Placebo-Controlled, Parallel-Group, 2-Arm (alirocumab Q2W, placebo, 1:1) • Study duration: 3 weeks screening, 96-weeks double-blind Tx period	• Primary: Change in Cambridge Neuropsychological Test Automated Battery (CANTAB) cognitive domain Spatial Working Memory (SWM) strategy score from baseline to week 96. • Secondary (efficacy): % change in calculated LDL-C, % change in Apo B, in non-HDL-C, in TC, in Lp(a), in HDL-C, in fasting TG, in Apo A-1, % of patients reaching calculated LDL-C <70 mg/dL (1.81 mmol/L) and LDL-C < 50mg/dL(1.29 mmol/L).	• SSD: Nov 2016 • DE: 2020

Alirocumab (anti-PCSK-9 mAb)
Neurocognitive Evaluation (2/2)

Immuno-inflammation | Diabetes
Oncology | **Cardiovascular**
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
Long Term Safety Study of Praluent **Regeneron** R727-CL-1609 NCT03694197	Phase 4 Evaluate the long term safety of PRALUENT in participants with heterozygous familial hypercholesterolemia (heFH) or non-familial hypercholesterolemia (FH) patients at high or very high cardiovascular risk who completed the neurocognitive function study (R727-CL-1532: NCT02957682)	1600	• Participants randomized into the neurocognitive function study (R727-CL-1532) who completed treatment and the end of study (EOS) visit with no premature or permanent discontinuation of study drug • Open Label, up to 192 weeks • Drug: Praluent	• Primary: Incidence of adverse events (AEs) after first administration of study drug through the last dose of study drug plus 2 weeks • Secondary: Changes in LDL-C and other lipid parameters, changes in gonadal steroid hormones	• SSD: Sep 2018 • DE: 2023

Fitusiran (siRNA targeting Antithrombin/AT3)
Hemophilia A & B (1/5)

Study	Description	Patients	Design	Endpoints	Status
Hemophilia A or B **LTE14762** **ALN- AT3SC-002** **NCT02554773**	Phase 1/2 Hemophilia A Hemophilia B Long term Safety and Efficacy of Fitusiran in patients with moderate or severe Hemophilia A or B	34	• For patients having participated in a previous fitusiran study • Single Group assignment, Open-label • Subjects are administred SC fitusiran once every month for the duration od the study	• Primary: incidence of treatment-emergent AEs, SAEs, and AEs leading to study drug discontinuation • Secondary: Changes in the N of Bleeding Event, the Amount of Factor VIII or Factor IX administered for the Tx of bleeding episodes, health-related QOL plasma levels of antithrombin and thrombin generation	• SSD: Sep. 2015 • DE: 2021

Fitusiran (siRNA targeting Antithrombin/AT3)
Hemophilia A & B (2/5)

Study	Description	Patients	Design	Endpoints	Status
ATLAS-INH **EFC14768** **ALN- AT3SC-003** **NCT03417102**	Phase 3 Hemophilia A Hemophilia B Efficacy and Safety of Fitusiran in patients with Hemophilia A or B, with Inhibitory Antibodies to Factor VIII or IX, who are not receiving prophylactic treatment	54	• In patients (Males ≥ 12 years old) suffering from severe hemophilia A or B with inhibitors, • Randomized, Parallel Assignment, Open-label • Fitusiran and active comparator (on demand bypassing agents)	• Primary: Annualized bleeding rate (ABR) [Time Frame: 9 months] • Secondary: 1) Annualized spontaneous bleeding rate [Time Frame: 9 months] 2) Annualized joint bleeding rate [Time Frame: 9 months] 3) Quality of Life (QOL) as measured by Haem-A-QOL Questionnaire score on a scale of 0-100 with higher scores representing greater impairment. [Time Frame: 9 months]	• SSD: Mar. 2018 • DE: 2020

Fitusiran (siRNA targeting Antithrombin/AT3)
Hemophilia A & B (3/5)

Study	Description	Patients	Design	Endpoints	Status
ATLAS-A/B **EFC14769** **ALN- AT3SC-004** **NCT03417245**	Phase 3 Hemophilia A Hemophilia B Efficacy and Safety of Fitusiran in patients with Hemophilia A or B, without Inhibitory Antibodies to Factor VIII or IX, who are not receiving prophylactic treatment	120	• In patients (Males ≥ 12 years old) suffering from severe hemophilia A or B without inhibitors, • Randomized, Parallel Assignment, Open-label • Fitusiran and active comparator (on demand Factor VIII or IX)	• Primary: Annualized bleeding rate (ABR) [Time Frame: 9 months] • Secondary: 1) Annualized spontaneous bleeding rate [Time Frame: 9 months] 2) Annualized joint bleeding rate [Time Frame: 9 months] 3) Quality of Life (QOL) as measured by Haem-A-QOL Questionnaire score on a scale of 0-100 with higher scores representing greater impairment. [Time Frame: 9 months]	• SSD: Jul. 2018 • DE: 2020

Fitusiran (siRNA targeting Antithrombin/AT3)
Hemophilia A & B (4/5)

Study	Description	Patients	Design	Endpoints	Status
ATLAS-PPX **EFC15110** **ALN- AT3SC-009** **NCT03549871**	Phase 3 Hemophilia A Hemophilia B Switching study to describe the Efficacy and safety of Fitusiran prophylaxis in Patients with Hemophilia A or B and previously receiving Factor or Bypassing Agent Prophylaxis	70	• For patients (males ≥ 12 years old) with severe hemophilia A or B with inihibitors. • Single Group assignment, Open-label • Tx: 7 months	• Primary: annualized bleeding rate (ABR) in the fitusiran efficacy period and the factor or BPA in prophylaxis period • Secondary: annualized spontaneous bleeding rate and annualized joint bleed rate in the fitusiran efficacy period and the factor or BPA in prophylaxis period, Quality of Life (QOL) measured by Haem-A-QOL Questionnaire, ABR in the fitusiran onset period (1 month), ABR in the fitusiran Tx period (7 months)	• SSD: Sept 2018 • DE: 2021

Fitusiran (siRNA targeting Antithrombin/AT3)
Hemophilia A & B (5/5)

Study	Description	Patients	Design	Endpoints	Status
LTE15174 **NCT03754790**	Phase 3 Hemophilia A Hemophilia B Long-term Safety and Efficacy of Fitusiran in Patients with Hemophilia A or B With or Without Inhibitory Antibodies to Factor VIII or X	252	• For patients (≥ 12 years old) with severe hemophilia A or B who have completed a Phase 3 fitusiran study, • Single Group assignment, Open-label • Study duration: up to 55 months per patient, including a screening period up to 30 days, an open label Tx period up to 48 months and a follow-up period up to 6 months	• Primary: AEs, • Secondary: annualized bleeding rate (ABR), annualized spontaneous bleeding rate and annualized joint bleed rate in the Tx period, Quality of Life (QOL) measured by Haem-A-QOL Questionnaire	• SSD: Jan. 2019 • DE: 2024

Sutimlimab (BIVV009 - Anti Complement C1s mAb) Cold Agglutinin Disease (CAgD) (1/2)

Study	Description	Patients	Design	Endpoints	Status
Cardinal **BIVV009-03** NCT03347396	Phase 3 Efficacy and Safety of BIVV009 in patients with Primary Cold Agglutinin Disease with a recent history of Blood Transfusion	24	• Patients suffering from primary cold agglutinin disease (CAD) with at least one blood transfusion within 6 months of enrollment • Open-label, Single Group assignment • Part A (required for registration): biweekly IV infusion of BIVV009 up to week 26 • Part B: long-term safety and durability of response extension phase for patients having completed Part A, BIVV009 dosing for up to 1 year after Part A LPO	• Primary (Part A): response rate (≥ 2g/dl increase in Hgb OR Hgb >12g/dl AND no transfusion required); • Secondary (Part A): change in bilirubin, change in FACIT-Fatigue Scale Score, change in LDH, number of transfusions and blood units and change in Hgb; • Part B: TEAEs, hemoglobin, bilirubin, FACIT-F, LDH, transfusion, haptoglobin, HRU.	• SSD: Nov. 2017 • DE: Part A: 2019, Part B: 2020

Sutimlimab (BIVV009 - Anti Complement C1s mAb) Cold Agglutinin Disease (CAgD) (2/2)

Study	Description	Patients	Design	Endpoints	Status
Cadenza **BIVV009-04** NCT03347422	Phase 3 Efficacy and Safety of BIVV009 in patients with Primary Cold Agglutinin Disease without a recent history of Blood Transfusion	40	• Patients suffering from primary cold agglutinin disease (CAD) with no blood transfusions in prior 6 months and no more than 1 blood transfusion in the prior 1 year • Randomized, double-blind, placebo controlled • Part A: biweekly IV infusion of BIVV009 or placebo (up to 26 weeks) • Part B: long-term safety and durability of response extension phase for patients having completed Part A. Blinded cross-over loading doses to allow all participants to receive BIVV009 while maintaining Part A blinding. BIVV009 dosing for up to 1 year after Part A LPO	• Primary (Part A); response rate (≥ 1.5g/dl increase in Hgb AND no transfusion required); • Secondary (Part A): change in Hgb, change in bilirubin, change in FACIT-Fatigue Scale Score, change in LDH, incidence of symptomatic anemia symptoms • Part B: TEAEs, hemoglobin, bilirubin, FACIT-F, LDH, transfusion, haptoglobin, HRU.	• SSD: Nov. 2017 • DE: : Part A: 2020, Part B: 2021

Sutimlimab (BIVV009 - Anti Complement C1s mAb)
Chronic Immune Thrombocytopenia (ITP)

Study	Description	Patients	Design	Endpoints	Status
TNT009-201/ BIVVOO9-201 NCT03275454	Phase 1 Safety, PK and PD of BIVV009 in patients with Chronic Immune Thrombocytopenia (ITP)	16	• Patients suffering from chronic ITP. Open-label, Single Group assignment • Part A: Bi-weekly IV infusion of BIVV009 up to 21 weeks • Part B: long-term treatment period (for 52 weeks) for patients who have had benefit from BIVV009 during Part A; patients undergo monitored washout from BIVV009 at end of Part A and enter Part B upon return of thrombocytopenia.	• Primary: TEAEs, premature study terminations, Clinical Laboratory Abnormalities • Efficacy endpoints: Part A & B: Change in platelet count; independence from additional ITP therapy; Number of patient who achieve complete response (CR), response (R); Duration of CR and R; Time to increased platelet count > 30, 50, and 100 x 10^9/L; number of patients with loss of CR, loss of R, • PK/PD endpoints: PK parameters, anti-drug antibodies, PD measures (Complement factor measures, thrombopoietin levels , immature platelet fraction, platelet autoantibody/autoantigen)	• SSD: Aug. 2017 • DE: 2019

ST400 (gene-editing technology)
Beta-thalassemia

Study	Description	Patients	Design	Endpoints	Status
Transfusion-dependent Beta-thalassemia (TDT) **ST-400-01** **NCT03432364**	Phase 1/2 Safety, Tolerability and Efficacy of ST400 Autologous Hematopoietic Stem Cell transplant for Tx of Transfusion-Dependent Beta-thalassemia (TDT)	6	• Patients with clinical diagnosis of TDT with at least 8 documented RBC transfusion events per year and confirmed diagnosis of beta-thalassemia (genetic testing) • Open-Label, Single Group Assignment, single dose	• Primary: AEs and SAEs • Secondary: change from baseline in Hb fractions measurements and % HbF, change in frequency and volume of packed red blood cells (PRBC) transfusions	• SSD: Mar. 2018 • DE: Primary: 2020, Full completion: 2022

BIVV003 (gene-editing technology)
Sickle Cell Disease (SCD)

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | **Rare Blood Disorders**
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
PRECIZN-1 **003SCD101** **NCT03653247**	Phase 1/2 Safety, Tolerability and Efficacy of BIVV003 for Autologous Hematopoietic Stem Cell Transplantation in Patients With severe Sickle Cell Disease	8	• Patients suffering from severe SCD • Open-Label, Single Group Assignment, single dose	• Primary: % of patients alive post-transplantation at D100, at week 52, at week 104, % of patients with successful engraftment, AEs, SAEs, • Secondary: CD34+HSPC yield from Plerixafor stem cell mobilization, % of patients with sufficient stem cell mobilization, yield of ZFN-edited IP, time to initial neutrophil recovery, time to platelet recovery, % of patients with maintenance of absolute neutrophil count ≥ 500/mcL, % of patients with maintenance of platelets count ≥ 50 000/mcL, change from baseline in HbF, in %F, in HbS, in REC, in LDH, in haptoglobin and bilirubin, QoL	• SSD: Jan. 2019 • DE: 2022

Caplacizumab - Cablivi™
Acquired Thrombotic Thrombocytopenic Purpura

Study	Description	Patients	Design	Endpoints	Status
Post-HERCULES **ALX0681-C302** **NCT02878603**	Phase 3 Evaluate the long-term safety and efficacy of caplacizumab, evaluate safety and efficacy of repeated use of caplacizumab and characterize the long-term impact of acquired Thrombotic Thrombocytopenic Purpura (aTTP).	104	• Prospective follow-up for adult patients (18 years and older) with acquired TTP who completed HERCULES • Single group assignment, open label • Study duration: Tx period (only for patients who experience a recurrence of aTTP during the study period): initial i.v. dose followed by daily s.c. injections for up to 6 months (max). Total study duration approximately 3 years	• Primary: proportion of subjects with TTP-related events, # of and time to TTP-related events, mortality rate, proportion of subjects with, # of and time to recurrence of disease, proportion of subjects with reported major thromboembolic events, # of and time to major thromboembolic events, cognitive function, quality of life assessment, immunogenicity, and AE	• SSD: Aug. 2016 • DE: 2020

Dengue Vaccine
Co-administration w/ Tdap booster
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT02992418	Phase 3 Study of a Tetravalent Dengue Vaccine Administered Concomitantly or Sequentially With Adacel® in Healthy Subjects	688	• Randomized, multicenter, open-label study in 688 subjects aged from 9 to 60 years	• Immunogenicity and safety of CYD dengue vaccine and Tdap vaccine when both vaccines are administered concomitantly or sequentially	• SSD: Dec. 2016 • DE: 2020

Dengue Vaccine
Different schedules
Asia Pacific, Latin America Regions

Study	Description	Patients	Design	Endpoints	Status
NCT02628444	Phase 2a Immunogenicity and Safety of 3-Dose and Booster Dose of Tetravalent Dengue Vaccine in Healthy Subjects 9 to 50 Years of Age	1050	• Two-stage, multi-national, multi-center, observer-blind, randomized, placebo-controlled Phase II immunogenicity and safety study of tetravalent dengue vaccine	• Immunogenicity and safety of 3-dose primary series and booster dose	• SSD: May. 2016 • DE: 2020

Dengue Vaccine
Booster dose
Latin America Region

Study	Description	Patients	Design	Endpoints	Status
NCT02623725	Phase 2b Study of a Booster Dose of a Tetravalent Dengue Vaccine in Subjects Who Previously Completed the 3-dose Schedule	251	• Multi-center, observer-blind, randomized, placebo-controlled, Phase II trial	• Immunogenicity and safety of a booster dose	• SSD: Apr. 2016 • DE: 2019

Rabies Vaccine
Verorab
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT01622062	Phase 3 Immunogenicity and Safety of Verorab® in a "One-week" Intradermal Post-exposure Prophylaxis Regimen	600	• Open-label, randomized, controlled, multi-center, multi-country trial	• Immunogenicity and safety of Verorab® in a "One-week" intradermal post-exposure prophylaxis regimen	• SSD: Jun. 2012 • DE: 2019

Meninge Vaccine
MenQuadTT
Greater Europe, Latin America, Asia Pacific Regions

Study	Description	Patients	Design	Endpoints	Status
NCT03205371	Phase 3 Immunogenicity and Safety of a Meningococcal Conjugate Vaccine Given Concomitantly With Other Vaccines in Toddlers	1183	• Open-label (immunology laboratory technicians will be blinded to group assignment), randomized, parallel-group, active-controlled, multi-center study	• Immunogenicity and safety	• SSD: Nov. 2016 • DE: 2019

Dengue Vaccine
Booster
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT02824198	Phase 2b Immunogenicity and Safety of a Tetravalent Dengue Vaccine Booster Injection in Subjects Who Previously Completed a 3-dose Schedule	260	• Multi-center, observer-blind, randomized, placebo-controlled, Phase II non-inferiority trial	• Immunogenicity and safety of a booster dose	• SSD: Jul. 2016 • DE: 2019

Dengue Vaccine
Co-administration w/ HPV
Latin America Region

Study	Description	Patients	Design	Endpoints	Status
NCT02979535	Phase 3b Immunogenicity and Safety of a Tetravalent Dengue Vaccine Administered Concomitantly or Sequentially With Cervarix®	480	• Randomized, open-label, multicenter study	• Immunogenicity and safety of a Tetravalent Dengue Vaccine administered concomitantly or sequentially with Cervarix®	• SSD: Nov. 2016 • DE: 2019

Dengue Vaccine
Co-administration w/ HPV
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT02993757	Phase 3b Immunogenicity and Safety of a Tetravalent Dengue Vaccine Administered Concomitantly or Sequentially With Gardasil®	528	• Randomized, open-label, multicenter study	• Immunogenicity and safety of a Tetravalent Dengue Vaccine administered concomitantly or sequentially with Gardasil®	• SSD: Dec. 2016 • DE: 2020

Dengue Vaccine
Latin America Region

Study	Description	Patients	Design	Endpoints	Status
NCT01374516	Phase 3 Study of a Novel Tetravalent Dengue Vaccine in Healthy Children and Adolescents Aged 9 to 16 Years in Latin America	20869	• Randomized, double-blind, controlled, multicenter	• Efficacy and safety	• SSD: Jun. 2011 • DE: 2018

AcP Primary
Africa and Middle East Regions

Immuno-inflammation | Diabetes
Oncology | Cardiovascular
Rare Diseases | Rare Blood Disorders
MS, Neuro, Gene therapy | Vaccines

Study	Description	Patients	Design	Endpoints	Status
NCT02817451	Phase 4 DTaP-IPV-HB-PRP-T Combined Vaccine as a Primary Series and a 2nd Year of Life Booster in HIV-Exposed Infected and Uninfected	100	• multicenter, open-label, two-arm study	• immunogenicity and safety of 3-dose primary series and booster dose	• SSD: Jul. 2016 • DE: 2020

Adacel+
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT02587520	Phase 1 Study of Tetanus Toxoid, Reduced Diphtheria Toxoid, and Acellular Pertussis Vaccine Adsorbed in Healthy Subjects	1364	randomized, modified double-blinded, multi-center, active comparator, dose and formulation ranging, step-down study,	• Safety and immunogenicity	• SSD: Oct, 2015 • DE: 2019

Dengue Vaccine
Asia Pacific

Study	Description	Patients	Design	Endpoints	Status
NCT02827162	Exploratory Phase Association of Host Genetics With Vaccine Efficacy and Study of Immune Correlates of Risk From a Tetravalent Dengue Vaccine	334	• Exploratory, single-center study	• Host generic analysis and correlate of protection	• SSD: Mar. 2016 • DE: 2018

Dengue Vaccine
Latin America, Asia Pacific Regions

Study	Description	Patients	Design	Endpoints	Status
NCT02948933	Epidemiology Phase Cohort Event Monitoring for Dengvaxia®, CYD-TDV Dengue Vaccine	30 000	• Observational	• Incidence of selected AEs and SAEs, occurrence and frequency of hospitalized dengue disease and SAEs leading to hospitalization or death	• SSD: Dec. 2016 • DE: 2025

Flu Vaccine
Latin America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03391193	Phase 3 Immunogenicity and Safety of a Multi-Dose Quadrivalent Influenza Vaccine	301	• Randomized, open-label, active-controlled, multi-center study in Mexico	• Immunogenicity and safety	• SSD: Dec. 2017 • DE: 2018

AcP Primary Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT00855855	Phase 4 Surveillance Program to Determine Product Specific Rates of Invasive Hib Disease	510 000	• Observational	• Surveillance for Hib disease.	• SSD: Feb. 2009 • DE: 2019

AcP Primary Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT01129362	Phase 4 Rates of Pertussis Disease Among Persons Receiving Pentacel® or Other Pertussis Vaccines	1 538	• Observational	• Occurrence of pertussis disease, as determined by the Wisconsin Division of Public Health (WDPH).	• SSD: May 2010 • DE: 2019

MenQuad TT Vaccine
North America Region, Latin America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03077438	Phase 3 Study of an Investigational Quadrivalent Meningococcal Conjugate Vaccine Administered in Children Aged 2 to 9 Years	999	• modified double-blind, randomized, parallel-group, active-controlled, multi-center trial	• Safety and immunogenicity	• SSD: Feb. 2017 • DE: 2019

MenQuad TT Vaccine
Greater Europe Region

Study	Description	Patients	Design	Endpoints	Status
NCT02955797	Phase 3 Immunogenicity and Safety of an Investigational Quadrivalent Meningococcal Conjugate Vaccine in Toddlers	918	Modified double-blind, randomized, parallel-group, active-controlled, multi-center trial	• Immunogenicity and safety	• SSD: Feb. 2017 • DE: 2018

Meninge Vaccine
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT02864927	Phase 4 Postmarketing Surveillance Study for Use of Menactra® in the Republic of Korea	600	• Open, Multi-center, observational, active safety surveillance study	• Occurrence of solicited and unsolicited events	• SSD: Jul. 2016 • DE: 2019

New Pertussis Vaccine
Latin America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03147898	Phase Epidemiology Observational Study Describing the Immune Profile Induced By Pertussis Vaccines	90	• Observational, multicenter trial	• Immune response to booster dose	• SSD: Apr. 2017 • DE: 2019

Flu seasonal Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT01945424	Phase Epidemiology Sanofi Pasteur Quadrivalent Influenza Vaccine (QIV) Pregnancy Registry	500	• Observational	• Pregnancy registry	• SSD: Nov. 2013 • DE: 2020

Japanese Encephalitis Vaccine
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT02933710	Phase 4 Postmarketing Surveillance Study for IMOJEV® in Republic of Korea	50	• Multi-center, open, observational, active safety surveillance study.	• Occurrence of solicited and unsolicited events	• SSD: Jul. 2016 • DE: 2019

MenQuad TT Vaccine
Booster
Greater Europe Region

Study	Description	Patients	Design	Endpoints	Status
NCT03476135	Phase 3 Immunogenicity and safety booster dose in subjects previously vaccinated as toddlers	91	• Open label, multicenter study to describe immune persistence of the priming dose and immuno and safety of booster dose	• Immunogenicity and safety	• SSD: Feb. 2018 • DE: 2019

MenQuad TT Vaccine
Co administration
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03537508	Phase 3 Safety and Immunogenicity for Infants, with co administration with routine pediatric vaccines	2475	Modified double blind study, randomized, parallel groups, active controlled, multicenter	• Immunogenicity and safety	• SSD: Apr. 2018 • DE: 2024

MenQuad TT Vaccine
Alternative schedules
Greater Europe Region

Study	Description	Patients	Design	Endpoints	Status
NCT03547271	Phase 3 Safety and ImmunogenIcity for alternative schedules in infants	1540	• Partially modified double blind, randomized, parallel group, active controlled, multi center	• immunogenicity and safety	• SSD: Dec. 2018 • DE: 2022

Flu seasonal Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03617523	Phase 4 Safety and immunogenicity Fluzone Quadrivalent, Flublock Quadrivlent and Fluzone High Dose	240	• Interventional, open label, randomized,	• Immunogenicity and safety	• SSD: Sep. 2018 • DE: 2019

MenQuad TT Vaccine
Latin America, Asia Pacific, Greater Europe Regions

Study	Description	Patients	Design	Endpoints	Status
NCT03630705	Phase 3 Safety and immunogenicity 3 dose schedule Quadrivalent Meningococcal conjugate vaccine	825	• Interventional, randomized, parallel assignement, active controlled multi center study	• Immunogenicity and safety	• SSD: Oct. 2018 • DE: 2022

MenQuad TT Vaccine
Latin America, North America

Study	Description	Patients	Design	Endpoints	Status
NCT03691610	Phase 3 Safety & Immunogenicity 2-dose Trial in Toddlers	940	• Interventional, modified double blind, Randomized, parrallel assignement active controlled multi center study.	• Immunogenicity and safety	• SSD: Oct. 2018 • DE: 2020

Flu QIV HD Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03698279	Phase 2b Safety and immunogenicity of Flu Quadrivalent HD 3 dose schedule in Pediatric population	700	• Interventional, Randomized, Sequential Assignment, modified double blind, multi center study	• Dose response, immunogenicity and safety	• SSD: Oct. 2018 • DE: 2020

Rabies Vaccine
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT03700242	Phase3 Immunogenicity and safety of HDCV with abbreviated pre-exposure regimens Trial	570	• Interventional, Randomized, Parallel Assignment,	• Immunogenicity and safety	• SSD: Sep. 2018 • DE: 2020