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APPENDICES
RESEARCH & DEVELOPMENT

April 27th, 2018

R&D Pipeline – New Molecular Entities(*)

Phase 1 (Total:14)		Phase 2 (Total:15)		Phase 3 (Total:6)	Registration (Total:2)
SAR439794 TLR4 agonist Peanut Allergy	**SAR442168**[3] BTK inhibitor Multiple Sclerosis	**SAR440340**(**) Anti-IL33 mAb Asthma	**SAR425899** GLP-1/GCG dual agonist Obesity/Overweight in T2D	**isatuximab** Anti-CD38 mAb 3L Relapsing Refractory MM (ICARIA)	**cemiplimab**[10](**) PD-1 inhibitor mAb Advanced CSCC (EU)
SAR408701 Maytansin-loaded anti-CEACAM5 mAb Solid Tumors	**SAR438335** GLP-1/GIP dual agonist Type 2 Diabetes	**SAR156597** IL4/IL13 bi-specific mAb Systemic Scleroderma	**mavacamten**[7](**) Myosin inhibitor Obstructive Hypertrophic Cardiomyopathy	**avalglucosidase alfa** Neo GAA Pompe Disease	**sotagliflozin**(**) Oral SGLT-1&2 inhibitor Type 1 Diabetes (U.S./EU)
SAR439459 anti-TGFβ mAb Advanced Solid Tumors	**SAR440181**[4](**) Myosin activation Dilated Cardiomyopathy	**GZ389988** TRKA antagonist Osteoarthritis	**SAR407899** rho kinase Microvascular Angina	**fitusiran**[9] siRNA targeting Anti-Thrombin Hemophilia A and B	
O **REGN3767**[1] Anti LAG-3 mAb Advanced Cancers	**SAR247799** S1P1 agonist Cardiovascular indication	R **SAR566658** Maytansin-loaded anti-CA6 mAb Triple Negative Breast Cancer	Combination **ferroquine / OZ439**(**) Antimalarial	**BIVV009** Anti Complement C1s mAb Cold Agglutinin Disease	
SAR439859 SERD Metastatic Breast Cancer	**Herpes Simplex Virus Type 2** HSV-2 vaccine	R **olipudase alfa** rhASM Acid Sphingomyelinase Deficiency[5]	**Tuberculosis** Recombinant subunit vaccine	**SAR341402** Rapid acting insulin Type 1/2 Diabetes	
BIVV001 rFVIIIFc – vWF – XTEN[2] Hemophilia A	**Respiratory syncytial virus** Infants Vaccines	O **SAR339375**[6] miRNA-21 Alport Syndrome	**HIV** Viral vector prime & rgp120 boost vaccine	**efpeglenatide**(**) Long-acting GLP-1 agonist Type 2 Diabetes	
UshStat® Myosin 7A gene therapy Usher Syndrome 1B		**venglustat** Oral GCS inhibitor Gaucher related Parkinson's Disease	**SP0232**[8] **mAb**(**) Respiratory syncytial virus Monoclonal Antibody		
SAR228810 Anti-protofibrillar AB mAb Alzheimer's Disease		**SAR422459** ABCA4 gene therapy Stargardt Disease			

R Registration Study

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

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

(1) Regeneron product for which Sanofi has opt-in right
(2) Recombinant Coagulation Factor VIII Fc – von Willebrand Factor – XTEN Fusion protein
(3) Also known as PRN2246
(4) Also known as MYK491
(5) Also known as Niemann Pick type B
(6) Regulus product for which Sanofi has opt-in right
(7) Also known as SAR439152 and MYK461
(8) Also known as MEDI8897
(9) Following the Alnylam/Sanofi strategic restructuring of the RNAi therapeutics rare disease alliance announced in January 2018, Sanofi now has global rights on fitusiran
(10) Also known as SAR439684 and REGN2810
(*) Data related to all studies published on clinicaltrials.gov
(**) 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:12)		Phase 3 (Total:16)		Registration (Total:4)
SAR439459 + cemiplimab(1)(**) Anti-TGFβ mAb + PD1 inhibitor mAb Advanced Solid Tumors	**dupilumab(**)** Anti-IL4Rα mAb Eosinophilic Esophagitis	**sotagliflozin(**)** SGLT 1 & 2 inhibitor Worsening Heart Failure in Diabetes	**dupilumab(**)** Anti-IL4Rα mAb Asthma 6 - 11 years old	**isatuximab** Anti-CD38 mAb 1-3L Relapsing Refractory MM (IKEMA)	**dupilumab(**)** Anti-IL4Rα mAb Asthma 12y+ (U.S./EU)
isatuximab Anti-CD38 mAb + CyBord(2) Newly Diagnosed MM	R **sarilumab(**)** Anti-IL6R mAb Polyarticular Juvenile Idiopathic Arthritis	**Rabies VRVg** Purified vero rabies vaccine	**dupilumab(**)** Anti-IL4Rα mAb Nasal Polyposis	**sotagliflozin(**)** Oral SGLT-1&2 inhibitor Type 2 Diabetes	**VaxiGrip® QIV IM** Quadrivalent inactivated Influenza vaccine 6 - 35 months
O **cemiplimab(1)(**) + REGN3767(3)** PD-1 inhibitor mAb + anti LAG-3 mAb Advanced Cancers	**sarilumab(**)** Anti-IL6R mAb Systemic Juvenile Arthritis	**Adacel+** Tdap booster	**Dupixent®(**)** Anti-IL4Rα mAb Atopic Dermatitis 12 – 17 years old	**Aubagio®** teriflunomide Relapsing Multiple Sclerosis - Pediatric	**PR5i** DTP-HepB-Polio-Hib Pediatric hexavalent vaccines (U.S.)
SAR439859 SERD + Palbociclib Metastatic Breast Cancer	R **cemiplimab(1)(**)** PD-1 inhibitor mAb Advanced Basal Cell Carcinoma	**Shan 6** DTP-HepB-Polio-Hib Pediatric hexavalent vaccine	**Dupixent®(**)** Anti-IL4Rα mAb Atopic Dermatitis 6 – 11 years old	**Lemtrada®** alemtuzumab Relapsing Remitting Multiple Sclerosis - Pediatric	**Fluzone® 0,5 mL QIV** Quadrivalent inactivated Influenza vaccine 6 months+
BIVV009 Anti Complement C1s mAb Immune Thrombocytopenia	**isatuximab + cemiplimab(1)(**)** Anti-CD38 mAb + PD-1 inhibitor mAb Relapsing Refractory MM		**Dupixent®(**)** Anti-IL4Rα mAb Atopic Dermatitis 6 months - 5 years old	**Praluent®(**)** Anti-PCSK9 mAb CV events reduction	
	isatuximab + cemiplimab(1)(**) Anti-CD38 mAb + PD-1 inhibitor mAb Advanced Malignancies		**cemiplimab(1)(**)** PD-1 inhibitor mAb 1L NSCLC	**Fluzone® QIV HD** Quadrivalent inactivated Influenza vaccine - High dose	
	venglustat Oral GCS inhibitor Gaucher Disease Type 3		**cemiplimab(1)(**)** PD-1 inhibitor mAb 2L Cervical Cancer	**Men Quad TT** Advanced generation meningococcal ACYW conjugate vaccine	
	venglustat Oral GCS inhibitor Fabry Disease		**isatuximab** Anti-CD38 mAb 1L Newly Diagnosed MM (IMROZ)	**Pediatric pentavalent vaccine** DTP-Polio-Hib Japan	

R Registration Study

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

- Immuno-inflammation
- Oncology
- Rare Diseases
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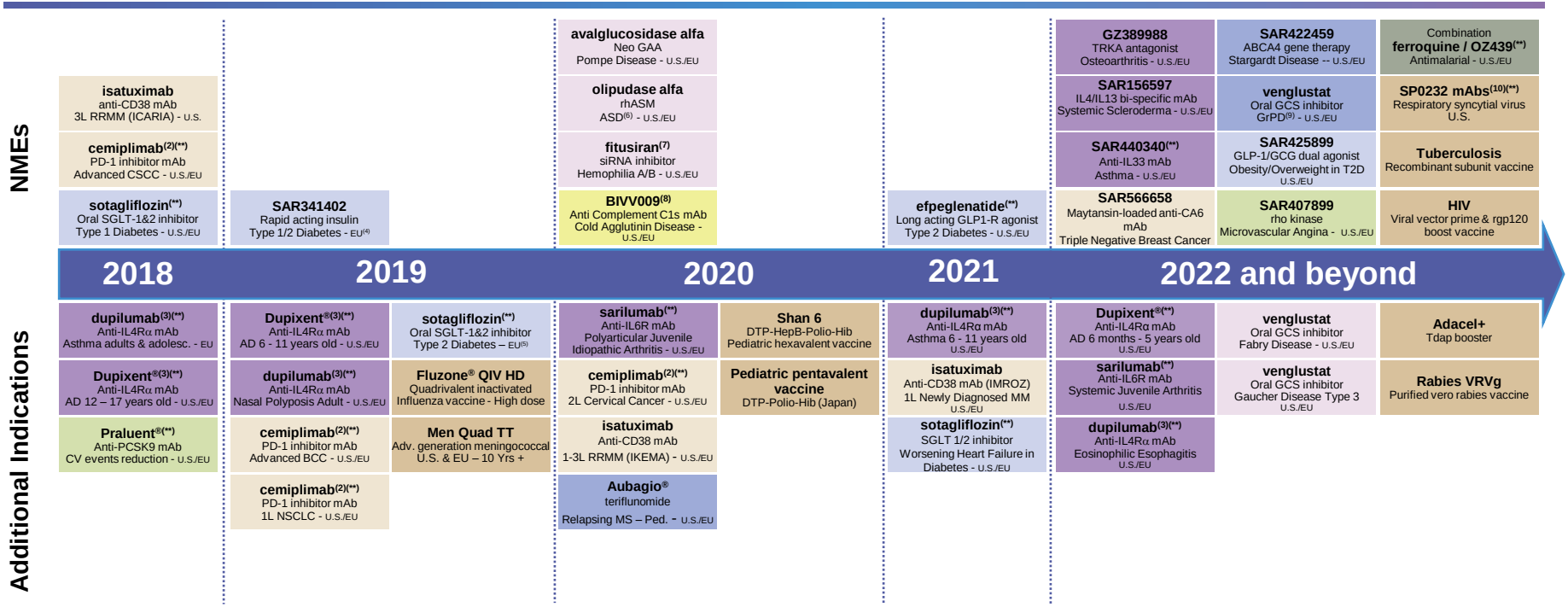
- Diabetes
- Cardiovascular & metabolism
- Infectious Diseases
- Vaccines

(1) Also known as SAR439684 and REGN2810
(2) Cyclophosmamide + bortezomib (Velcade®) + dexamethasone
(3) Regeneron product for which Sanofi has opt-in right
(*) Data related to all studies published on clinicaltrials.gov
(**) Partnered and/or in collaboration - Sanofi may have limited or shared rights on some of these products

Expected Submission Timeline(1)



(1) Excluding Phase 1 - Data related to all studies published on clinicaltrials.gov
(2) Also known as SAR439684 and REGN2810
(3) Also known as SAR231893
(4) Submission strategy for the U.S. under evaluation
(5) Submission for the U.S. expected in 2020
(6) Acid Sphingomyelinase Deficiency
(7) Following the Alnylam/Sanofi strategic restructuring of the RNAi therapeutics rare disease alliance announced in January 2018, Sanofi now has global rights on fitusiran
(8) Currently operating as separate entities. Reported dates are based on prior Bioverativ disclosure of study completion date
(9) Gaucher Related Parkinson's Disease
(10) Also known as MEDI8897
(**) 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, Gene therapy
- Diabetes
- Cardiovascular & metabolism
- Infectious Diseases
- Vaccines

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Pipeline Movements Since Q4 2017

	Additions to the pipeline		Removals from the pipeline
Phase 1	**BIVV001** rFVIIIFc – vWF – XTEN[1] Hemophilia A		**lumasiran**[3] Investigational RNAi therapeutic Primary Hyperoxaluria Type 1 (PH1)
	BIVV009 Anti Complement C1s mAb Immune Thrombocytopenia		
Phase 2	**SAR440340**(**) Anti-IL33 mAb Asthma	**isatuximab + cemiplimab**[2](**) Anti-CD38 mAb + PD-1 inhibitor mAb Advanced Malignancies	
	isatuximab + cemiplimab[2](**) Anti-CD38 mAb + PD-1 inhibitor mAb Relapsing Refractory MM		
Phase 3	**BIVV009** Anti Complement C1s mAb Cold Agglutinin Disease		
Registration	**cemiplimab**[2](**) PD-1 inhibitor mAb Advanced CSCC (EU)	**Fluzone® 0,5 mL QIV** Quadrivalent inactivated Influenza vaccine 6 months+	
	sotagliflozin(**) Oral SGLT-1&2 inhibitor Type 1 Diabetes (U.S./EU)		

(1) Recombinant Coagulation Factor VIII Fc – von Willebrand Factor – XTEN Fusion protein
(2) Also known as SAR439684 and REGN2810
(3) In March 2018 Sanofi Genzyme declined its opt-in for the development and commercialization of lumasiran (ALN-GO1)
(**) Partnered and/or in collaboration – Sanofi may have limited or shared rights on some of these products

R&D Pipeline Summary – Total Projects(1)

	Phase 1	Phase 2	Phase 3	Registration	TOTAL
Immuno-inflammation	1	6	5	1	13
Oncology	8	4	5	1	18
Rare Diseases	0	4	2	0	6
Rare Blood Disorders	2	0	1	0	3
Multiple Sclerosis, Neurology, Gene therapy	3	2	2	0	7
Diabetes	1	2	3	1	7
Cardiovascular Diseases	2	2	1	0	5
Infectious Diseases	0	1	0	0	1
Vaccines	2	6	3	3	14
TOTAL	**19**	**27**	**22**	**6**	

46 **28**

74 **Total Projects**

(1) Includes 2 Phase 1 products and 1 Phase 2 product for which Sanofi has Opt-in rights but has not yet exercised these rights

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Clinical Trials Appendices

List of abbreviations

AE	Adverse Events
APO	Apolipoprotein
BOR	Best Overall Response
CB	Clinical Benefit
CNS	Central Nervous System
CR	Complete Response
CRR	Complete Response Rate
CT	Computed Tomography
CV	Cardiovascular
DE	Data Expected
DCR	Disease Control Rate
DLT	Dose-Limiting Toxicity
DOD	Duration Of Disease
DOR	Duration Of Response
EASI	Eczema Area and Severity Index
FPC	Fasting Plasma Glucose
IAE	Incidence of Adverse Events
IAR	Infusion Associated Reaction
IC	Investigator's Choice
IGA	Investigator's Global Assessment
IMID	Immunomodulatory Drug
ITT	Intent To Treat
LP	Lipoprotein
MRI	Magnetic Resonance Imaging
MTD	Maximum Tolerated Dose
N	Number
NC	Nasal Congestion/obstruction
NNT	Number Needed to Treat
OS	Overall Survival
ORR	Overall Response Rate
PD	Pharmacodynamics
PI	Proteasome Inhibitor
PFS	Progression-Free Survival
PK	Pharmacokinetic
PPG	Postprandial Glucose
PRO	Patient Reported Outcome
QNW	Every N Weeks
QNM	Every N Months
QOL	Quality Of Life
RECIST	Response Evaluation Criteria in Solid Tumors
SAE	Serious Adverse Events
SDMT	Symbol Digit Modalities Test
SMPG	Self Monitored Plasma Glucose
SSD	Study Start Date
TC	Total Cholesterol
TEAE	Treatment Emergent Adverse Events
TSS	Total Symptom Score
TG	Triglycerides
TTP	Time To Progression
TTR	Time To Response
TX	Treatment
VGPR	Very Good Partial Response

Dupilumab (anti-IL4Rα mAb)
Asthma 1/3

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	• For patients coming from DRI12544, PDY14192, EFC13579, EFC13691 studies: dupilumab loading dose sc on Day 1, followed by 1x dose Q2W added to current controller medications • Open-label, max. 3 weeks screening and 108 weeks Tx	• Primary: N and % of patients experiencing any TEAE • Secondary: Safety	• SSD: Jul. 2014 • DE: 2019

Dupilumab (anti-IL4Rα mAb)
Asthma 2/3

Study	Description	Patients	Design	Endpoints	Status
EXPEDITION ASTHMA PDY14192 NCT02573233	Phase 2a Evaluation of dupilumab's effects on airway inflammation in patients with asthma	42	• Randomized, double-blind, parallel, placebo-controlled Study, 5 to 6 weeks screening, 12 weeks Tx, 12 weeks post Tx	• Primary: Change from baseline in N of inflammatory cells and in mucin-stained area in the bronchial submucosa per mm^2 • Secondary: Safety, Tolerability, Immunogenicity of dupilumab compared to placebo	• SSD: Jan. 2016 • DE: 2018

Dupilumab (anti-IL4Rα mAb)
Asthma 3/3

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

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	294	• 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)
Atopic Dermatitis (AD)

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 • Non-Randomized, Parallel Assignment, Open label extension study	• Primary: Incidence and rate of 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. 2015 • DE: 2023
Pediatrics (12 to 17 years) AD R668-AD-Reg 1526 NCT03054428	Phase 3 A study to investigate the efficacy and safety of dupilumab monotherapy in patients 12 to 17 years of age, with moderate-to-severe AD	240	• Pediatric patients (12 to 17 years old) with moderate-to-severe AD • A randomized, double-blind, placebo-controlled, 3-arm: dupilumab dose 1, dupilumab dose 2, placebo	• Primary: % of patients with IGA 0 to 1 (on a 5-point scale), % of patients with EASI-75 • Secondary: % change in EASI score	• SSD: Apr. 2017 • DE: 2018

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

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	• Primary: PK, TEAEs, SAEs • Secondary: SEAs, TEAEs, % chanhe in EASI score, Change in children's Dermatology Quality of Life Index	• SSD: Dec. 2017 • DE: 2022
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	240	• 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: Dec. 2017 • DE: 2019

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

Study	Description	Patients	Design	Endpoints	Status
Autoinjector R668-AD-1608 NTC03050151	Phase 1 Study of Dupilumab Auto-injector Device When Used by Patients With Atopic Dermatitis	176	• Part A: Patients with moderate-to-severe AD will be randomized to receive dupilumab (dose 1) by auto-injector (AI) device or prefilled syringe. • Part B: Once part A is completely enrolled, part B will randomize patients with moderate-to-severe AD to receive dupilumab (dose 2) by auto-injector (AI) device or prefilled syringe	• Primary: Number and type of validated AI device-associated PTFs during the treatment period by actual number of injections • Secondary: Number of patients with an AI device associated PTF, Number and type of AI device-associated PTCs, Number of patients with an AI device associated PTC, Type of AI device-associated failed drug deliveries, Number of patients with an AI device-associated failure to deliver dose, PK	• SSD: Mar. 2017 • DE: 2018
Open-Label R668-AD-1225 NCT01949311	Phase 3 Open-Label study of Dupilumab in patients with Atopic Dermatitis	2000	• 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: 2018

Dupilumab (anti-IL4Rα mAb)
Nasal Polyposis (NP)

Study	Description	Patients	Design	Endpoints	Status
NP SINUS-24 EFC14146 NCT02912468	Phase 3 Evaluation of dupilumab in patients with bilateral NP on a background of mometasone furoate nasal spray	276 finally included	• Patients with bilateral sinonasal polyposis that despite prior Tx with systemic corticosteroids have an endoscopic bilateral NPS with a score at least of 5 over 8 • Randomized, double-blind, placebo-controlled study, 4 weeks run-in, 24 weeks Tx, 24 weeks post-Tx	• Primary: NC symptom severity score based on the patient daily morning assessment & by endoscopy, Sinus opacifications as assessed by CT • Secondary: TSS, Loss of smell, Sinus opacification	• SSD: Dec. 2016 • DE: 2018
LIBERTY NP SINUS-52 EFC14280 NCT02898454	Phase 3 Evaluation of dupilumab in patients with bilateral NP on a background of mometasone furoate nasal spray	448 finally included	• Patients with bilateral sinonasal polyposis that despite prior Tx with systemic corticosteroids have an endoscopic bilateral NPS with a score at least of 5 over 8 • Randomized, double-blind, placebo-controlled study, 4 weeks run-in, 52 weeks Tx, 12 weeks post-Tx, 3-arm, dupilumab dose regimen 1, dupilumab dose regimen 2, placebo	• Primary: NC symptom severity score based on the patient daily morning assessment & by endoscopy, Sinus opacifications as assessed by CT • Secondary: TSS, Loss of smell, Sinus opacification	• SSD: Dec. 2016 • DE: 2018

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 Tx 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 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, 92-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: Sep. 2016 • DE: 2018
Systemic JIA Children & Adolescents DRI13926 NCT02991469	Phase 2b Dose-finding study of sarilumab in children and adolescents with Systemic Juvenile Idiopathic Arthritis (sJIA)	36	• In children and adolescents, aged 1 to 17 years, with sJIA • Open-label, sequential, ascending, repeated dose finding study, 4-week screening, 12-week Tx, 92- 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: Dec. 2017 • DE (1st part) (1): 2018

(1) Final Data Collection date for primary outcome measure

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: Dec. 2016 • DE (1st part) (1): 2018

(1) Final Data Collection date for primary outcome measure

SAR440340 (Anti-IL33 mAb)

Asthma – single agent and in combination with dupilumab

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

Study	Description	Patients	Design	Endpoints	Status
Asthma R3500-AS-1619 NCT02999711	Phase 1 Safety and tolerability of multiple ascending subcutaneous doses of SAR440340 in adult patients with Moderate Asthma	23	• Randomized, double-blind, Placebo-controlled, Multiple ascending dose study of the safety • Cohort 1: SAR440340 low dose or placebo • Cohort 2 : SAR440340 medium dose or placebo	• Primary: Incidence of TEAEs after repeat subcutaneous administration, severity of TEAEs • Secondary: Concentration-time profile of REGN3500 after repeat subcutaneous administration, Immunogenicity, % change in total from baseline forced expiratory volume	• SSD: Feb. 2017 • DE: 2018
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

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	240	• 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 every • 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: Apr. 2018 • DE: 2019

SAR439794 (TLR4 agonist) Immunomodulator

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

Study	Description	Patients	Design	Endpoints	Status
Peanut Allergy TDR14287 NCT03463135	Phase 1 Safety,Ttolerability and Pharmacodynamics of SAR439794 in Peanut Allergic Adult Patients	44	• Randomized, Double-blind, Placebo-controlled, 3 Arms • Repeated Sublingual daily Administration of SAR439794 or placebo • Total study duration per participant: approximately from 15 to 18 weeks (core study) from screening until end-of-study visit, and 2 phone calls at Week 26 and Week 52 after the last IMP dose	• Primary: Incidence of AEs • Secondary: PD parameters (peanut-specific serum IgG levels, peanut-specific serum IgE levels, SkinPrick test)	• SSD: Mar. 2018 • DE: 2020 (completion)

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

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

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	346	• 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)
Multiple Myeloma (MM)

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	60	• 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)

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	89	• 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

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

Study	Description	Patients	Design	Endpoints	Status
Bortezomib Combination **RRMM** 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 Part) (1): 2018

(1) Final Data Collection date for primary outcome measure

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

Study	Description	Patients	Design	Endpoints	Status
RRMM TED14154 NCT02514668	Phase 1 Safety, PK and Efficacy of isatuximab in patients with Relapsed/Refractory MM	64	• 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: 2020

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

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	42	• 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: 2018

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

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	105	• 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: 2021

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

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	300	• 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): 2018

(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 (1st Part)(1): 2020

(1) Final Data Collection date for primary outcome measure

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

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

Study	Description	Patients	Design	Endpoints	Status
IMROZ NDMM 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 or patient's refusal of transplant • Randomized, Open-label, Parallel assignment • IVRd arm (Isatuximab/bortezomib/lenalidomide /dexamethasone) • VRd arm (Bortezomiblenalidomide /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, OS, TTP, DOR, PFS on next line of therapy (PFS2), AE, PK, Immunogenicity, QOL	• SSD: 2017 • DE (1st Part) (1): 2021

(1) Final Data Collection date for primary outcome measure

Isatuximab (anti-CD38 mAb) combination cemipimab (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)	134	• 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	• SSD: 2018 • DE: 2021

(1) Final Data Collection date for primary outcome measure

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: cemilplimab 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)

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 montherapy in Japanese patients with AM	14	• Histologically or cytologically confirmed diagnosis of malignancy with no alternative standard-of-care therapeutic option • Single Group assignment, Open-label	• Primary: TEAEs cemiplimab PK parameters • Secondary: Immunogenicity against cemiplimab	• SSD: Sep. 2017 • DE: 2019

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

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	301	• 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: 2019 (Primary completion)

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	30	• For Histologically confirmed diagnosis of stage III (unresectable) or stage IV melanoma 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 (1st Part) (1): 2018

(1) Final Data Collection date for primary outcome measure

Cemiplimab (PD-1 inhibitor)
Head and Neck

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

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	• SSD: Jul. 2017 • DE (1st Part) (1): 2018

(1) Final Data Collection date for primary outcome measure

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

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)	182	• 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: ORR (96 weeks), Groups 1 and 3: RECIST version 1.1 will be used to determine ORR, Group 2: Clinical response criteria will be used to determine ORR • Secondary: Investigator Assessments of ORR, DOR, DOD, PFS, OS, CRR	• SSD: May 2016 • DE: 2019
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 | Infectious disease
Rare Blood Disorders | Vaccines
MS, Neuro, Gene therapy

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	• SSD: July 2017 • DE (1st Part) (1): 2018

(1) Final Data Collection date for primary outcome measure

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

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

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	300	• 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, 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: 2021

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 aptients 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 stage IV disease who received no prior systemic Tx for recurrent or metastatic NSCLC • Randomized, Open-label, Parallel assignment • Arm 1: Standard of care (SOC) • Arm 2: cemiplimab + SOC • 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

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

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

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 mNSCLC with tumors expressing PD-L < 50%	201	• For histologically or cytologically documented squamous or non squamous NSCLC with stage IIIB and not candidates for definitive chemoradiation or stage IV. Patients immunotherapy naïve and having received one prior cytoxic regimen. • 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, QoL, immunogenicity, hair pigmentation, tumor burden, tumor volume, PK, markers	• SSD: May 2017 • DE: 2021

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

Study	Description	Patients	Design	Endpoints	Status
CC R2810-ONC-1676 NCT03257267	Phase 3 Cemiplimab vs. therapy of IC chemotherapy in Recurrent or Metastatic Platinum-Refractory CC	436	• Patients with recurrent or metastatic platinum-refractory CC treated with either REGN2810 or IC chemotherapy • Randomized, Open-label, Parallel assignment, Tx cycle 6 weeks, Planned Tx for up to 96 weeks	• Primary: OS • Secondary: PFS, ORR, DOR, QOL	• SSD: Oct. 2017 • DE (1st Part)(1): 2020

(1) Final Data Collection date for primary outcome measure

SAR566658 (maytansin loaded anti-CA6 mAb)
Triple Negative Breast Cancer (TNBC)

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

Study	Description	Patients	Design	Endpoints	Status
mTNBC ACT14884 NCT02984683	Phase 2b Efficacy and safety of SAR566658 Tx in patients with CA6 Positive Metastatic TNBC	62	• Patients with Measurable Metastatic TNBC, with CA6-positive disease • Randomized, Open-label, Parallel assignment; Tx cycle 3 weeks • Part 1: SAR566658 will be given as Dose 1 (cohort 1) and Dose 2 (cohort 2) at Day 1 and Day 8 every 3 weeks intravenously (dose selection) • Part 2: SAR566658 will be given as Dose 1 or Dose 2 (depending on dose level selected from part 1) at Day 1 and Day 8 every 3 weeks intravenously (efficacy of the selected dose)	• Primary: ORR • Secondary: DCR, DOR, PFS, TTP, Impact of ocular primary prophylaxis on the incidence of keratopathies, Potential immunogenicity of SAR566658	• SSD: Mar. 2017 • DE: 2019

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

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

Study	Description	Patients	Design	Endpoints	Status
AST **Monotherapy and combination with cemiplimab** TCD14678 NCT03192345	Phase 1/1b PK, PD and Anti-tumor activity of SAR439459 Monotherapy and in combination with cemiplimab in adult patients with AST	130 expected	• Patients with histologically confirmed, advanced unresectable or metastatic solid tumor • Randomized, Open-label, Parallel assignment • Part 1A: SAR439459 monotherapy escalating doses/14-day cycle • Part 2A: SAR439459 monotherapy/14-day cycle with the previously recommended dose • Part 1B: SAR439459 escalating dose + cemiplimab standard dose /14-day cycle • Part 2B: SAR439459 at previously recommended dose + cemiplimab standard dose / 14-day • Escalation periods non randomized followed par expansion periods randomized	• Primary: DLTs (Part 1), ORR (Part 2) • Secondary: Safety, Immunogenicity, PFS, TTP, PK	• SSD: Jun. 2017 • DE: 2020

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

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

Study	Description	Patients	Design	Endpoints	Status
First-in-Human TED13751 NCT02187848	Phase 1 Phase 2 PK and antitumor activity of SAR408701 in patients with AST	233 expected	• 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 lung adenocarcinoma 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 lung adenocarcinoma (Lung bis) at MTD • Arm 7: SAR408701 expansion cohort colorectal cancer (CRC-L) at MTD	• Primary: MTD, Anti-tumor response RECIST • Secondary: Safety, Immunogenicity, PK	• SSD: Sep. 2014 • DE: 2019

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

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

Study	Description	Patients	Design	Endpoints	Status
Japanese patients **Monotherapy and Combination** TCD15054 NCT03324113	Phase 1 Safety and PK of SAR408701 Monotherapy and in combination with other anti-tumor drug in Japanese patients with Advanced Malignant Solid Tumors	27	• Patients with malignant solid tumor • Non-Randomized, Open-label, Sequential assignment • Phase 1 : SAR408701 monotherapy escalating doses/ 4 weeks • Phase 1B: SAR408701 at MTD in combinations with other anti-tumor drugs, 4 weeks	• Primary: DLTs, Phase 1 and 1B • Secondary: Safety, Immunogenicity, PK, Plasma CEACAM5 levels, Anti-tumor response RECIST	• SSD: Oct. 2017 • DE: 2020

SAR439859 (SERD)
Breast cancer

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

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 B • 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

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

Study	Description	Patients	Design	Endpoints	Status
COMET **Late Onset** EFC14028 NCT02782741	Phase 3 To compare efficacy and safety of Enzyme Replacement therapies avalglucosidase alfa and alglucosidase alfa in patients with Late-onset PD who have not been previously treated for 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 from baseline in percent predicted forced vital capacity (%FVC) in upright position • Secondary: Change from baseline in six-minute walk test scores, maximal inspiratory / expiratory pressure in upright position, hand-held dynamometry measurement of lower extremity muscle strength in Quick Motor Function Test scores, 12- Item Short-form health survey scores	• SSD: Nov. 2016 • DE (1st Part)(1): 2019

(1) Final Data Collection date for primary outcome measure

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

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

Study	Description	Patients	Design	Endpoints	Status
Mini-COMET Infantile Onset ACT14132 NCT03019406	Phase 2 To assess safety and efficacy of avalglucosidase alfa in Pediatric patients with infantile-onset PD previously treated With alglucosidase alfa	20	• In Patients with Infantile-onset PD treated with alglucosidase alfa 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 • Secondary: PK parameters, Change from baseline in Gross Motor Function (GMF) Measure-88 Test, Change from baseline 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 (1st Part)(1): 2019

(1) Final Data Collection date for primary outcome measure

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

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 in patients with PD	24	• In patients with PD who previously completed a avalglucosidase alfa study [adult, senior] • Non-randomized, Open-label, Parallel 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 immunoglobulin G (IgG) antibodies, and neutralizing antibody formation in IgG seropositive 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

Patisiran(1) (siRNA targeting TTR)
Hereditary ATTR (hATTR) Amyloidosis

Study	Description	Patients	Design	Endpoints	Status
Global Open-Label Extension (OLE) Study ["APOLLO-OLE"] LTE14730 ALN-TTR02-006 NCT02510261	Phase 3 An open-label extension study evaluating the long-term safety and efficacy of patisiran in patients with hereditary transthyretin mediated amyloidosis (hATTR) with polyneuropathy who completed the Phase 2 OLE and Phase 3 APOLLO studies	211	• For eligible patients who completed the Phase 2 OLE and Phase 3 APOLLO studies • Long-term use of patisiran • Single group assignement, Open-label	• Primary: Safety and tolerability of long-term dosing of patisiran as measured by the proportion of subjects with AE leading to discontinuation of study drug • Secondary: Changes from baseline in neurologic impairment assessed using the Neuropathy Impairment Score (NIS), the Modified NIS (mNIS +7) composite score, the NIS+7 QOL [(QOL-DN) and EuroQOL (EQ-5D)], autonomic and motor function, disability, nutritional status, serum TTR lowering	• SSD: Jul. 2015 • DE: 2019

(1) Following the Alnylam/Sanofi strategic restructuring of the RNAi therapeutics rare disease alliance announced in January 2018, Alnylam now has global rights on patisiran and Sanofi will receive royalties based on net sales of patisiran.

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

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

Study	Description	Patients	Design	Endpoints	Status
Hemophilia A or B LTE14762 ALN- AT3SC-002 NCT02554773	Phase 1/2 Hemophilia A and Hemophilia B 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	• Primary: % of patients experiencing 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

(1) Following the Alnylam/Sanofi strategic restructuring of the RNAi therapeutics rare disease alliance announced in January 2018, Sanofi now has global rights on fitusiran.

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

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	54	• In patients 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: Annualized spontaneous bleeding rate [Time Frame: 9 months], Annualized joint bleeding rate [Time Frame: 9 months], 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: Apr. 2018 • DE: 2019

(1) Following the Alnylam/Sanofi strategic restructuring of the RNAi therapeutics rare disease alliance announced in January 2018, Sanofi now has global rights on fitusiran.

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

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

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	120	• In patients 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: Annualized spontaneous bleeding rate [Time Frame: 9 months], Annualized joint bleeding rate [Time Frame: 9 months], 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: Apr. 2018 • DE: 2019

(1) Following the Alnylam/Sanofi strategic restructuring of the RNAi therapeutics rare disease alliance announced in January 2018, Sanofi now has global rights on fitusiran.

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

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 ASD	36	• Randomized, Double-blinded, Placebo-controlled, Parallel assignment • Total study duration for one patient at least 3 years up to 5 years and 3 months [2-month screening, 52-week double-blind Tx period, 4-year and 1 month open label extension period with olipudase	• 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, Change in fatigue severity as measured by item 3 of the Brief Fatigue Inventory scale, Change in pain severity as measured by item 3 of the Brief Pain Inventory scale, Change in dyspnea severity as measured by the Functional Assessment of Chronic Illness Therapy dyspnea tool	• SSD: Jun. 2016 • DE (1st Part)[2]: 2019

(1) Non-neurological manifestations of ASMD
(2) Final Data Collection date for primary outcome measure

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

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: Jun. 2015 • DE: 2019

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

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: 5 years	• 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: 2023

Venglustat (GCS inhibitor)
Fabry disease (FD)

Study	Description	Patients	Design	Endpoints	Status
FABRY LONG-TERM LTS14116 NCT02489344	Phase 2 Long-term safety, PD, and exploratory efficacy of venglustat in Tx-naïve adult male patients with FD	8	• Male patients with FD who previously completed study ACT13739 • Open-label, Single group Assignment • Total study duration for one patient: up to 31 months	• Primary: Safety profile, Clinically significant changes in laboratory parameter, and physical examinations • Secondary: Change from baseline in plasma globotriaosylceramide (GL-3), plasma lyso GL-3, Change from baseline in plasma glucosylceramide (GL 1), Urine GL-3	• SSD: Jul. 2015 • DE: 2018

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

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

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 2 part-ttmt 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: Mar. 2017 • DE (1st Part)(1): 2021

(1) Final Data Collection date for primary outcome measure

BIVV009 (former TNT009) (Anti Complement C1s mAb) Cold Agglutinin Disease (CAgD)

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	20	• Patients suffering from primary cold agglutinin disease (CAgD) with at least one blood transfusion within 6 months of enrollment • Open-label, Single Group assignment • Part A (required to registration) IV infusion of BIVV009 up to week 26 • Part B for patients having completed Part A, BIVV009 up to 1 year after LPO in Part A	• Primary: Response rate (no transfusion required and ≥ 2g/dl increase in Hgb) in Part A; TEAEs in Part B • Secondary: Change in bilirubin, Change in FACIT, Fatigue Scale Score, Change in lactate dehydrogenase, N of transfusions and blood units and change in Hgb	• SSD: Nov. 2017 • DE (1st Part)(1): 2019

(1) Final Data Collection date for primary outcome measure

BIVV009 (former TNT009) (Anti Complement C1s mAb) Cold Agglutinin Disease (CAgD)

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

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 (CAgD) • Randomized, double-blind, placebo controlled • Part A, IV infusion of BIVV009 or placebo (up to 26 weeks) (required for registration) • Part B: response extension phase , blinded cross-over loading doses to allow all participants to receive BIVV009 while maintaining Part A blinding (up to 1 year after Part A LPO)	• Primary: Response rate in Part A; TEAEs in Part B • Secondary: Change in Hgb, change in bilirubin, change in FACIT, Fatigue Scale Score, Change in lactate dehydrogenase, Incidence of symptomatic anemia symptoms	• SSD: Nov. 2017 • DE (1st Part)(1): 2019

(1) Final Data Collection date for primary outcome measure

BIVV009 (former TNT009) (Anti Complement C1s mAb) Chronic Immune Thrombocytopenia (ITP)

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

Study	Description	Patients	Design	Endpoints	Status
TNT009-201 NCT03275454	Phase 1 Safety, PK and PD of BIVV009 in patients with Chronic Immune Thrombocytopenia (ITP)	16	• Patients suffering from chronic immune thrombocytopenia refractory to at least 2 prior therapies • Open-label, Single Group assignment • IV infusion of BIVV009 up to week 21	• Primary: TEAEs, Premature study terminations, Clinical Laboratory Abnormalities • Secondary: Change in platelet count, Independence from additional ITP therapy, % of participants with CR, Response, No response, Loss of CR, Loss of response, PK parameters, Anti-drug antibodies, Complement factors measures, Thrombopoietin levels, Immature platelets fraction, Antibodies against platelet antigens	• SSD: Aug. 2017 • DE: 2019

BIVV001 (rFVIIIFc-vWF-XTEN[(1)])
Hemophilia A

Study	Description	Patients	Design	Endpoints	Status
EXTEN-A **242HA101** NCT03205163	Phase 1 Phase 2 Safety, Tolerability and PK of a single dose regimen of Single dose of BIVV001 in Previously Treated Adults With Severe Hemophilia A	18	• Open-Label, Sequential Assignment • Low-Dose cohort: low dose of rFVIII[(2)] , washout of at least 72-hour and one single low dose of BIVV001 (25 IU/kg) • High-Dose cohort: single high dose of rFVIII, washout of at least 96-hour and a single high dose of BIVV001 (65 IU/kg)	• Primary: Annual Bleed Rate (ABR), Clinically significant laboratory abnormalities • Secondary: PK of rFVIII, Incremental recovery of FVIII, PK of BIVV001, Incremental activity of BIVV001	• SSD: Jul. 2017 • DE: 2019

(1) Recombinant Coagulation Factor VIII Fc – von Willebrand factor – XTEN fusion protein
(2) Recombinant Coagulation Factor VIII

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: 2019

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 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: 2012 • DE: 2034

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

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	28	• 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: Apr. 2012 • DE: 2020
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	28	• 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: Dec. 2012 • DE: 2035

Venglustat (GCS inhibitor)
GBA-PD

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: Jan. 2017 • DE: 2021

Insulin glargine / lixisenatide
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
LIXILAN-G **EFC13794** NCT02787551	Phase 3 Efficacy and safety of lixilan vs. GLP-1 receptor agonist in patients with type 2 Diabetes not controlled on GLP-1 RAs + OADs, with an extension period	500	• Patients with T2DM • Randomized, Open-label, Active Controlled, Parallel-group • Active comparator: Liraglutide/Exenatide/Exenatide ER/Albiglutide/Dulaglutide, Metformin, pioglitazone and SGLT2 inhibitor if taken prior to the study continued • 1st period: up to 2 weeks screening, 26-week Tx period and 3 to 9 days follow-up post Tx • Extension period 26-week extension after the 26-week Tx for the lixiLan arm only, 3-day follow-up post extension	• Primary: Change from baseline in HbA1c • Secondary: % of participants reaching HbA1c targets, Change from baseline in FPG, in 7-point SMPG, in 2-hour PPG during standardized meal test, in blood glucose excursion during standardized meal test , in body weight, Symptomatic hypoglycemia, Safety, % of patients requiring rescue therapy	• SSD: Jul. 2016 • DE: 2018

Insulin glargine / lixisenatide
Type 2 Diabetes Mellitus (T2DM) - Japan

Study	Description	Patients	Design	Endpoints	Status
LIXILAN JP-O1 **EFC14112** NCT02749890	Phase 3 Efficacy and safety of lixilan compared to lixisenatide on top of OADs in Japanese patients with T2DM with an extension period	318	• Japanese Patients with T2DM • Randomized, Open-label, Active Controlled, Parallel-group, 2- Tx arm • Active comparator: lixisenatide • Background therapy with OADs (except dipeptidyl-peptidase-4 inhibitor) should be continued during the Tx period • Study duration: approximately 55 weeks: up to 2-week screening, 26-week Tx period, 26-week safety extension Tx period and 3-day post Tx follow-up	• Primary: Change from baseline in HbA1c • Secondary: % of patients reaching HbA1c <7% or ≤6.5%, Change from baseline in FPG, in 7 point SMPG, % of patients reaching HbA1c <7% with no body weight gain, Change from baseline in body weight, % of patients requiring a rescue therapy, Change in daily dose of lixiLan for the combination group, N of hypoglycemic events, N of AE, Measurement from baseline of anti-lixisenatide antibodies and of anti-insulin antibodies	• SSD: May 2016 • DE: 2018

Insulin glargine / lixisenatide
Type 2 Diabetes Mellitus (T2DM) - Japan

Study	Description	Patients	Design	Endpoints	Status
LIXILAN JP-L **EFC14113** NCT02752412	Phase 3 Efficacy and safety of lixilan compared to insulin glargine with Metformin in Japanese patients with T2DM inadequately controlled on Basal Insulin and Oral Antidiabetic Drugs	534	• Japanese Patients with T2DM • Randomized, Open-label, Active Controlled, Parallel-group, 2- Tx arm • Active comparator: insulin glargine • Background therapy: Metformin will be continued • Study duration: approximately 41 weeks: up to 2-week screening, 12-week run-in, 26-week randomized Tx period and 3-day post Tx follow-up	• Primary: Change from baseline in HbA1c • Secondary: % of patients reaching HbA1c <7% or ≤6.5%, Change from baseline, in 2-hour PPpG, in blood glucose excursion during standardized meal test, in 7-point SMPG profiles (each time point and average daily value), in body weight, in FPG, in daily dose of insulin glargine, % of patients reaching HbA1c <7% with no body weight gain/no documented symptomatic hypoglycemia, % of patients requiring a rescue therapy, hypoglycemic events , AE, Measurement from baseline of anti-lixisenatide antibodies and of anti-insulin antibodies from baseline	• SSD: Aug. 2016 • DE: 2018

Insulin glargine / lixisenatide
Type 2 Diabetes Mellitus (T2DM) - Japan

Study	Description	Patients	Design	Endpoints	Status
LIXILAN JP-O2 **EFC14114** NCT02752828	Phase 3 Efficacy and safety of lixilan compared to Insulin Glargine on top of OADs in Japanese patients with T2DM	534	• Japanese Patients with T2DM • Randomized, Open-label, Active Controlled, Parallel-group, 2- Tx arm • Active comparator: insulin glargine • Background therapy with OADs (except dipeptidyl-peptidase-4 inhibitor) should be continued during the Tx period • Study duration: approximately 29 weeks: up to 2-week screening, 26-week randomized open-label Tx period and 3-day post Tx follow-up	• Primary: Change from baseline in HbA1c • Secondary: % of patients reaching HbA1c <7% or ≤6.5%, Change from baseline, in 2-hour PPG, in 7 point SMPG profiles during standardized meal test, in body weight % of patients reaching HbA1c <7% with no body weight gain/no documented symptomatic hypoglycemia, % of patients requiring a rescue therapy, N of AE, N of hypoglycemic events, Measurement from baseline of anti-lixisenatide antibodies and of anti-insulin antibodies from baseline	• SSD: Jun. 2016 • DE: 2018

Insulin glargine / lixisenatide
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
LIXILAN-D **LPS14860** NCT03434119	Phase 3 Efficacy and safety of SOLIQUA100/33™ compared to Lantus™ in ethnically/racially diverse patients with T2DM	1200	• Adult patients with T2DM not achieving glycemic control (i.e. HbA1c between 7.5% and 10% (inclusive)) on basal insulin and OADs, and who are Hispanics of any race, non-Hispanic black/African Americans or non-Hispanic Asians • Randomized, open-label, active-controlled, multi-center • Study duration: 29 weeks (2-week screening, 26-week randomized open-label tx period, 3-day post tx follow-up)	• Primary: Change from baseline to Week 26 in HbA1c (%) (overall and within each ethnic/racial subgroup evaluated) • Secondary: (within each ethnic/racial subgroup evaluated): Patients with HbA1c<7% at week 26; change in 2-hour post-prandial glucose (PPG); 2-hour glucose excursion; change in body weight; change in insulin glargine dose at Week 26, Hypoglycemia events, AE	• SSD: Feb. 2018 • DE: 2019

Lantus – Toujeo
U300 Type 1 Diabetes Mellitus (T1DM) - Children

Study	Description	Patients	Design	Endpoints	Status
EDITION JUNIOR **EFC13957** NCT02735044	Phase 3 Efficacy and safety of a new formulation of insulin glargine (U300) and Lantus® injected once daily in children and adolescents Age 6 - 17 years with T1DM with a 6-month safety extension period	450	• Children: 6 to 17 years old with T1DM • Randomized, Open-label, Parallel-group, 2- Tx arm • Active comparator: insulin glargine • Study duration: approximately 58 weeks: up to 2-week screening, 6-month comparative Tx period , 6-month comparative extension period and 4-week post Tx follow-up	• Primary: Change from baseline in HbA1c • Secondary: % of patients with HbA1c values of <7.5% and % of patients with FPG of ≤130 mg/dL (7.2 mmol/L) without any episode of severe and/or documented (SMPG <54 mg/dL; 3.0 mmol/L) symptomatic hypoglycemia during the last 3 months of the main 6-month randomized period, Change from baseline in FPG, Change from baseline in 24-hour mean plasma glucose and in variability of 24-hour mean plasma glucose based on 8-point SMPG profiles, % of patients with hypoglycemia, % of patients with hyperglycemia with ketosis, % of patients with AE	• SSD: April 2016 • DE: 2018

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: Dec. 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: Dec. 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: Mar. 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: Sept. 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: Sept. 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 2), in SBP for patients with baseline SBP ≥130 mmHg (for sotagliflozin doses 1/2), in SBP for all patients (for sotagliflozin dose 1), % of patients with Hemoglobin A1c (HbA1c) <7.0% (for sotagliflozin doses 1/2), % of patients with Hemoglobin A1c (HbA1c) <6.5% (for sotagliflozin doses 1/2), % of patients with AE	• SDD: Oct. 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	10500	• 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 N of heart failure events, CV death , 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 singlr-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), 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: Nov. 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	• 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: To compare the metabolic and gastrointestinal PD effects of an 8 weeks treatment with sotagliflozin once daily (QD) to an 8 weeks treatment to empagliflozin QD in mild or moderate hypertensive T2DM patients on a stable treatment regimen with metformin and an angiotensin converting enzyme (ACE) inhibitor or Angiotensin Receptor Blocker (ARB) under standardized diet conditions • Secondary: To compare the renal and cardiovascular PD effects of an 8 weeks treatment with sotagliflozin QD to an 8 weeks treatment to empagliflozin QD in mild or moderate hypertensive T2DM patients on a stable treatment regimen with metformin and an ACE inhibitor or ARB, To evaluate the safety and tolerability of an 8 weeks QD treatment with sotagliflozin or empagliflozin in mild to moderate hypertensive T2DM patients on a stable treatment with metformin and an ACE inhibitor or ARB, To evaluate the PK profile of sotagliflozin in steady state conditions	• SSD: Apr. 2018 • 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

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

Study	Description	Patients	Design	Endpoints	Status
T2 DM **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: Change in HbA1c (%) from Baseline to Week 56, Change from Baseline to Weeks 30 and 56 in Fasting plasma glucose and 7-point SMPG profiles, HbA1c <7.0% at Week 30 and Week 56 (Y/N), Change from Baseline to Weeks 30 and 56 in body weight, Safety and immunogenicity	• SSD: Dec. 2017 • DE: 2020

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	500	• 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

SAR341402 (Rapid Acting Insulin)
Type 1 Diabetes Mellitus (T1DM)

Study	Description	Patients	Design	Endpoints	Status
PDY15083 NCT03436498	Phase 1 Safety assessment of SAR341402 and NovoLog® used in continuous subcutaneous infusion for Type 1 Diabetes Mellitus Patients	30 (evaluable)	• Multi-center, randomized, open-label, two-sequence, two-treatment, 2-period, active-controlled, 2 x 4 weeks cross-over study assessing the safety of SAR341402 and NovoLog® used in CSII in patients with Type 1 diabetes mellitus (T1DM) • Patients will be randomized 1:1 to sequences of either SAR341402/ NovoLog® or NovoLog®/SAR341402. After completion of the first 4 weeks of treatment, patients on SAR341402 will be switched to NovoLog® and patients on NovoLog® will be switched to SAR341402 • The study duration for each patient will be approximately 10 weeks, including a 2-week screening period, 2 treatment periods of 4 weeks each, and 1-day post-treatment safety follow-up period	• Primary: To assess the safety of SAR341402 and NovoLog® when used in external insulin pumps in terms of the number of patients with infusion set occlusions • Secondary: To assess the safety of SAR341402 and NovoLog® when used in external pumps in terms of unexplained hyperglycemia, To assess the safety of SAR341402 and NovoLog® when used in external pumps in terms of: Intervals for infusion set changes, N of patients with insulin pump for "non-delivery" alarm, Patient observation of infusion set occlusion, AE and SAE, N of patients with hypoglycemic events (according to ADA Workgroup on hypoglycemia)	• SSD: May 2018 • DE: 2018

SAR425899 (GLP-1R/GCGR)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
T2DM **EFC13940** NCT02973321	Phase 2b Safety and efficacy of SAR425899 in overweight to obese patients with T2DM	270	• Overweight and obese patients with T2DM for at least 3 months before the screening visit. On diet/exercise and/or Tx with metformin (stable dose of ≥1500 mg/day or maximal tolerated dose) for at least 3 months prior to screening • Randomized, Double-blind, Placebo-controlled, Dose-ranging (SAR425899 3 doses, placebo) • Active comparator: liraglutide • Study duration: approximately 30-week: 3-week screening period at site, 26-week Tx period, 3-day follow-up period	• Primary: Change in HbA1c (%) • Secondary: Change in body weight, % of patients achieving predefined HbA1c targets of <7%, % of patients achieving predefined HbA1c targets of <6.5%, % of patients achieving ≥5% body weight loss, % of patients achieving ≥10% body weight loss, PK parameters	• SSD: Dec. 2016 • DE: 2018

SAR425899 (GLP-1R/GCGR)
Type 2 Diabetes Mellitus (T2DM) Overweight/Obese

Study	Description	Patients	Design	Endpoints	Status
Overweight to Obese patients with T2DM **TDR15516** NCT03414736	Phase 1 Safety and tolerability of SAR425899 in overweight to obese patients and T2DM patients not requiring anti-diabetic pharmacotherapy with an optional 6-month safety extension period.	60	• Overweight and obese patients and T2DM not requiring anti-diabetic pharmacotherapy; HbA1c ≤ 7.0%. • Randomized, open-label, 3 arm study with SAR425899 (3 different dose escalation regimens) • Study duration approximately 12 weeks for main study (up-to 3-week screening period, 8-week treatment period, 3-day follow-up period) and approximately 9 months for those participating in the 6 month safety extension (12 weeks main part and 6 month extension)	• Primary: Frequency and severity of GI adverse events (main study and 6 month extension) • Secondary: Change in body weight, fasting plasma glucose and HbA1c (main study and 6 month extension)	• SSD: Jan. 2018 • DE: 2018

SAR425899 (GLP-1R/GCGR)
Type 2 Diabetes Mellitus (T2DM) Overweight/Obese

Study	Description	Patients	Design	Endpoints	Status
Overweight to Obese patients with T2DM **PDY15264** NCT03350191	Phase 1 A PET/CT tracer study to investigate SAR425899 binding to the liver and pancreas in overweight to obese patients with T2DM	14	• Overweight and obese patients with T2DM diagnosed at least 1 year prior to study inclusion • Open-label study with treatment duration of 20 days • Total study duration approximately 4-7 weeks (including 21 day screening period, 20 day treatment period, and 7-day follow up period)	• Primary: % glucagon receptor occupancy in the liver (change of glucagon receptor tracer binding in the liver with SAR425899 between day 1 and day 20 • Secondary: % GLP-1 receptor occupancy in the pancreas (Change of GLP-1 receptor tracer binding in the pancreas with SAR425899 between day 1 and day 17)	• SSD: Dec. 2017 • DE: 2018

SAR425899 (GLP-1R/GCGR)
Overweight to Obese Subjects

Study	Description	Patients	Design	Endpoints	Status
Overweight to Obese Subjects **PDY15012** NCT03376802	Phase 1 Effect of SAR425899 on Energy Expenditure in Obese Subjects	30	• Randomized, double-blind, placebo-controlled study to asses the effect of repeated doses of SAR425899 on energy expenditure and safety in overweight to obese subjects • Total study duration of 5-8 weeks (including 21 day screening period, 7 day run-in period, 19-day treatment period, and 3-day follow-up period)	• Primary: sleep energy expenditure (change from baseline to day 19) • Secondary: total daily energy expenditure, resting energy expenditure and basal energy expenditure (change from baseline to day 19)	• SSD: Feb. 2018 • DE: 2018

SAR425899 (GLP-1R/GCGR)
Non-Alcoholic SteatoHepatitis (NASH)

Study	Description	Patients	Design	Endpoints	Status
NASH **ACT15067** NCT03437720	Phase 2 Efficacy and Safety of SAR425899 for the treatment of Non-Alcoholic SteatoHepatitis (NASH)	126	• A multi-center, double-blind, randomized, placebo-controlled study to assess the efficacy and safety of SAR425899 for the treatment of NASH in overweight or obese patients with NASH and with or without T2DM • Total study duration: approximately 64 weeks (including an 8 week screening period, a 52 week treatment period, and a 4 week follow-up period)	• Primary: Percentage of patients with resolution of NASH (ballooning component of NAS =0) without worsening of fibrosis score at week 52 • Secondary: Percentage of patients who achieve status of no hepatocyte ballooning with lobular inflammation score of 0 or 1 without worsening of fibrosis score at week 52; % of patients who achieve an improvement of fibrosis by at least 1 stage without worsening of the hepatocyte ballooning component of NAS at week 52; change from baseline to week 52 in the overall NAS	• SSD: to be confirmed • DE: to be confirmed

Alirocumab (anti-PCSK-9 mAb)
CV Events Reduction

Study	Description	Patients	Design	Endpoints	Status
ODYSSEY Outcomes EFC11570 NCT01663402	Phase 3 Evaluate the effect of alirocumab on the occurrence of CV Events in patients who have recently experienced an Acute Coronary Syndrome (ACS)	18600	• Patients recently (< 52 weeks) hospitalized for ACS • Randomized, Double-Blind, Placebo-Controlled, Parallel-Group • Study duration: max 64 months: up to 4 months run-in period, 60 months randomized Tx period	• Primary: Time from randomization to first occurrence of one of the following clinical events: CHD death, any non-fatal MI, fatal and non-fatal ischemic stroke, unstable angina requiring hospitalization • Secondary: Time to the first occurrence of any CHD event, major CHD event, any CV event, composite of all cause mortality/non-fatal MI/non-fatal ischemic stroke, all cause mortality, Change from baseline in blood lipids and LP levels	• SSD: Nov. 2012 • DE: 2018

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

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	30	• 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) • Backgroung 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 • 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: 2018

Alirocumab (anti-PCSK-9 mAb)
HeFH & non-FH Japan

Study	Description	Patients	Design	Endpoints	Status
ODYSSEY NIPPON EFC14305 NCT02584504	Phase 3 Efficacy and safety of alirocumab in patients with Hypercholesterolemia not adequately controlled with non-statin lipid modifying therapy or the lowest strength of statin	159	• Japanese Patients with hypercholesterolemia heFH or non-familial hypercholesterolemia receiving non statin LP modifying therapies (LMTs) or the lowest strength of statin • Randomized, Double-blind, Placebo-controlled, Parallel Group, 3-arm (alirocumab dose 1, alirocumab dose 2, placebo) • Backgroung therapies: stable and lowest-dose statin therapy or stable non-statin LMTs (eg, atorvastatin, fenofibrate, bezafibrate, ezetimibe) including diet therapy • Study duration: approximately 71 weeks: 4-week run-in period, 3-week screening period, 12-week double-blind Tx period, 52-week open-label Tx period	• Primary: % change in calculated LDL-C using all LDL-C values regardless of adherence to Tx • Secondary: % change in calculated LDL-C using all LDL-C values during the efficacy Tx period, % change in calculated LDL-C, % change in Apo-B, non-HDL-C, in TC, % of patients reaching LDL-C goal, % change in Lp(a), HDL-C, fasting TG, Apo A-1	• SSD: Sep. 2016 • DE: 2018

Alirocumab (anti-PCSK-9 mAb)
LDL Lowering China

Study	Description	Patients	Design	Endpoints	Status
ODYSSEY EAST EFC13889 NCT02715726	Phase 3 Efficacy and safety of alirocumab vs. ezetimibe in Asia in High CV risk patients with Hypercholesterolemia not adequately controlled with their statin therapy	600	• 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 (Week -3) • Randomized, Double-blind, Parallel Group, 2-Arm • Active comparator: ezetimibe • Background therapies: atorvastatin, rosuvastatin, or simvastatin continued during the course of the trial • Study duration: max 35 weeks: 3-week screening period, 24-week randomized Tx period, 8-week follow-up period	• Primary: % change in calculated LDL-C in the intent-to-treat (ITT) population • Secondary: % change in calculated LDL-C in the modified ITT (mITT) population, % 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)	• SSD: Aug. 2016 • DE: 2018

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

Study	Description	Patients	Design	Endpoints	Status
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 • 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)
Neurocognitive Evaluation

Study	Description	Patients	Design	Endpoints	Status
Neurocognitive Evaluation **Regeneron** R727-CL-1532 NCT02957682	Phase 3 Evaluate the effect of alirocumab on Neurocognitive function in patients with HeFH and non-HeFH at high and very high cardiovascular risk	2100	• 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 (safety) at week 96 in the CANTAB domains and compared to baseline raw scores: Paired Associates Learning, Reaction Time, SWM, global composite • 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

SAR439152 (Myosin inhibitor)
Obstructive Hypertrophic Cardiomyopathy (oHCM)

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

Study	Description	Patients	Design	Endpoints	Status
PIONEER-HCM **MyoKardia collaboration** MYK-461-004 NCT02842242	Phase 2 Efficacy, PK, PD, Safety and tolerability of SAR439152/MYK-461 in subjects with Symptomatic Hypertrophic Cardiomyopathy and Left Ventricular Outflow Tract Obstruction	21	• Patients with HCM (hypertrophied and non-dilated left ventricle in absence of systemic or other known cause), with LV wall thickness ≥ 15 mm at time of initial diagnosis or ≥ 13 mm with a positive family history of HCM • Open-label, Pilot, Single Group Assignment	• Primary: Change in post-exercise peak LVOT gradient from baseline to Week 12 • Secondary: Not provided	• SSD: Oct. 2016 • DE: 2018

SAR439152 (Myosin inhibitor)
Obstructive Hypertrophic Cardiomyopathy (oHCM)

Study	Description	Patients	Design	Endpoints	Status
PIONEER-OLE **MyoKardia collaboration** MYK-461-008 NCT03496168	Phase 2 An Open-Label Extension Study of Mavacamten (MYK-461) in Adults With Symptomatic Obstructive Hypertrophic Cardiomyopathy Previously Enrolled in Study MYK-461-004 (PIONEER)	20	• Open label	• Frequency and severity of AE and SAE	• Initiate patient dosing 1H 2018 • DE: 2H 2020

SAR439152 (Myosin inhibitor)
Obstructive Hypertrophic Cardiomyopathy (oHCM)

Study	Description	Patients*	Design	Endpoints(1)	Status
EXPLORER-HCM **MyoKardia Collaboration** MYK-461-005 NCT03470545	Phase 3 A Randomized, Double Blind, Placebo Controlled Clinical Study to Evaluate Mavacamten (MYK-461) in Adults With Symptomatic Obstructive Hypertrophic Cardiomyopathy	200-250	• Randomized, double-blind, placebo-controlled	• Post-exercise LVOT gradient, Change in exercise capacity (peak VO2), NYHA functional class, LVEF, Dyspnea symptom score, NT-proBNP	• Regulatory update Q2 2018 • Initiate patient dosing in Q2 2018 • DE: Undisclosed

(1) Pending Finalization

SAR439152 (Myosin inhibitor)
Non-obstructive Hypertrophic Cardiomyopathy (nHCM)

Study	Description	Patients	Design	Endpoints	Status
MAVERICK-HCM **MyoKardia Collaboration** MYK-461-006 NCT03442764	Phase 2 A Randomized, Double-blind, Placebo-controlled, Concentration-guided, Exploratory Study of Mavacameten in Patients With Symptomatic Non-Obstructive Hypertrophic Cardiomyopathy (nHCM) and Preserved Left Ventricular Ejection Fraction	60	• This is a multicenter, exploratory, randomized, double-blind study of the administration of mavacamten in 60 participants with symptomatic nHCM randomized to receive a 16-week course of mavacamten doses titrated to achieve 1 of 2 target drug concentrations. Dose adjustments will be based on PK parameters	• Primary: Safety and tolerability; Secondary: exercise capacity by peak oxygen uptake (peak VO2), changes in NYHA, diastolic and systolic function by echocardiography, symptoms and quality of life measures, NT pro-BNP levels	• SSD: March 2018 • DE: 2019

SAR407899 (Rho.kinase inhibitor)
Microvascular Angina (MA)

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

Study	Description	Patients	Design	Endpoints	Status
Rho-Kinase ACT14656 NCT03236311	Phase 2a Effects of SAR407899 in patients with MA and/or Persistent Stable Angina despite angiographically successful elective Percutaneous Coronary Intervention	78	• Patients with Symptomatic stable angina pectoris (typical or atypical symptoms with at least once weekly episodes); ECG evidence of ischemia with ST-segment depression during a symptom limited exercise test or non-invasive evidence of ischemia • Randomized, Double-blind, Placebo-controlled Parallel Arm Dose Titration over 4-week administration	• Primary: Assess effects of SAR407899 on coronary vasomotor function using coronary flow reserve assessed by 13N-ammonia or 82rubidium PET scan • Secondary: Assess effects of SAR407899 on QOL using Seattle Angina Questionnaire physical limitation domain (SAQ-PL) safety with a focus on hypotension and orthostatic hypotension plasma concentrations	• SSD: Oct. 2017 • DE: 2018

SAR247799 (S1P1 agonist)
Endothelial Function in patients with T2DM

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

Study	Description	Patients	Design	Endpoints	Status
Endothelial Function PDY15286 NCT03462017	Phase 1 Study to Assess the Pharmacodynamic Effects of Repeated Oral Doses of SAR247799 on Endothelial Function in Male and Female Patients With Type 2 Diabetes Mellitus	108	• Type-2 diabetes patients with % flow mediated dilation <7% at screening • Treatment groups:SAR247799, placebo, sildenafil (active comparator) • Treatment duration: 28 days	• Primary: Absolute change from baseline in the % flow-mediated dilation index of brachial artery • Secondary: Change from baseline in peak flow induced by acetylcholine iontophoresis measured by laser doppler perfusion monitoring, Safety, PK	• SSD: Mar. 2018 • DE: 2018

SAR440181 (Myosin activator)
Dilated cardiomyopathy (DCM)

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

Study	Description	Patients	Design	Endpoints	Status
MYK-491 Phase 1b SAD in DCM Patients **MyoKardia Collaboration** **MYK-491-003** NCT03447990	Phase 1 Randomized, Double-blind, Crossover, Placebo-controlled, Adaptive Design Study of Safety, Tolerability, Preliminary Pharmacokinetics, and Pharmacodynamics of Single Ascending Oral Doses of MYK-491 in Patients With Stable Heart Failure	12	• This is a randomized, crossover, double-blind, placebo-controlled, two cohort, sequential ascending single dose study. All patients will receive placebo and active doses of MYK-491 (low, med and/or high)	• Primary: Frequency and severity of treatment-emergent AE and SAE	• SSD: Feb. 2018 • DE: Nov. 2018

Ferroquine – Artefenomel / OZ439
Malaria

Study	Description	Patients	Design	Endpoints	Status
FALCI **DRI12805** NCT02497612	Phase 2 Efficacy, Safety, Tolerability and PK of a single dose regimen of ferroquine with artefenomel (OZ439) in adults and children with Uncomplicated Plasmodium Falciparum Malaria	662	• Patients from 6 months to 70 years suffering from mono-infection by P. falciparum • Randomized, Double-blind, Parallel Assignment • 4 doses of ferroquine associated to 1 dose of artefenomel according to age and body weight • Study duration: up to 67 days for each patient	• Primary: % of patients with Polymerase Reaction Chain (PCR)-adjusted Adequate Clinical and Parasitological Response (ACPR) • Secondary: Time to re-emergence, Time to recrudescence, Parasite clearance time, % of patients with PCR - crude ACPR, SAE, AESI, TEAE, % of patients with PCR - adjusted ACPR	• SSD: Jul. 2015 • DE: 2019

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

Flu Vaccine
Fluzone HD-QIV HV
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03282240	Phase 3 Safety and Immunogenicity of High-Dose Quadrivalent Influenza Vaccine in Participants ≥65 Years in the US	2616	• Ph3 randomized ,modified double blind, active controlled, multi center	• Safety, immunogenicity, consistency	• SSD: Sep. 2017 • DE: 2018

Flu Vaccine
Fluzone HD-QIV HV (Japan)

Study	Description	Patients	Design	Endpoints	Status
NCT03233217	Phase 1/2 Safety and Immunogenicity of High-Dose Quadrivalent Influenza Vaccine in Patients ≥65 Years	175	• Ph1/2 randomized, modified double blind, multi center	• Safety and immunogenicity	• SSD: Sep. 2017 • DE: 2018

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	1200	• 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

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

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

Rabies Vaccine
Purified Vero Rabies
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03145766	Phase 2 Immunogenicity and Safety of a Purified Vero Rabies Vaccine	320	• Multicenter, observer-blind, controlled, randomized, Phase II study	• Immunogenicity and safety	• SSD: Apr. 2017 • DE: 2018

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: 2020

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: 2019

Dengue Vaccine
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT01373281	Phase 3 Study of a Novel Tetravalent Dengue Vaccine in Healthy Children Aged 2 to 14 Years in Asia	10275	• Randomized, double-blind, controlled, multicenter	• Efficacy and safety	• SSD: Jun. 2011 • DE: 2018

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

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

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

Study	Description	Patients	Design	Endpoints	Status
NCT02587520	Phase 1 Study of Tetanus Toxoid, Reduced Diphtheria Toxoid, and Acellular Pertussis Vaccine Adsorbed in Healthy Subjects	1350	• Randomized, modified double-blinded, multi-center, active comparator, dose and formulation ranging, step-down study	• Safety and immunogenicity	• SSD: Oct, 2015 • DE: 2018

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	364	• 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	30000	• 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: 2026

Flu seasonal Vaccine
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT03344029	Phase 4 Immunogenicity and Safety of the Shenzhen Trivalent Inactivated Influenza Vaccine Versus a Trivalent Influenza Vaccine Comparator in Chinese Subjects 18 to 59 Years	1600	• Blind-observer, monocenter, randomized, comparative study	• Immunogenicity and safety	• SSD: Nov. 2017 • DE: 2018

Flu Vaccine
Latin America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03391193	Phase 3 Immunogenicity and Safety of a Multi-Dose Quadrivalent Influenza Vaccine	360	• Randomized, open-label, active-controlled, multi-center study in Mexico	• Immunogenicity and safety	• SSD: Dec. 2017 • DE: 2019

Flu seasonal Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03308825	Phase 4 Safety and Immunogenicity of Fluzone® Quadrivalent and Fluzone® High-Dose, Influenza Vaccines	240	• Multi-center, open-label trial	• Safety and immunogenicity	• SSD: Sep. 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	510000	• 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: 2018

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	1 000	• Modified double-blind, randomized, parallel-group, active-controlled, multi-center trial	• Safety and immunogenicity	• SSD: Feb. 2017 • DE: 2018

MenQuad TT Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT02842853	Phase 3 Immune Lot Consistency, Immunogenicity, and Safety of an Investigational Quadrivalent Meningococcal Conjugate Vaccine	3 344	• Modified double-blind, randomized, parallel-group, active-controlled, multi-center study	• Immune Lot Consistency, Immunogenicity, and Safety	• SSD: Jul. 2016 • DE: 2018

MenQuad TT Vaccine
North America Region, Latin America Region

Study	Description	Patients	Design	Endpoints	Status
NCT02842866	Phase 3 Immunogenicity and Safety of an Investigational Quadrivalent Meningococcal Conjugate Vaccine in Adults 56 Years and Older	910	• Modified double-blind, randomized, parallel-group, active-controlled, multi-center trial	• Immunogenicity and safety	• SSD: Jul. 2016 • DE: 2018

MenQuad TT Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT02199691	Phase 2 Immunogenicity and Safety of an Investigational Quadrivalent Meningococcal Conjugate Vaccine in Healthy Adolescents	1 715	• Open-label administration, randomized, parallel-group, controlled, multi-center study	• Immunogenicity and safety	• SSD: Jul. 2014 • DE: 2018

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	1 200	• 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	120	• 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	1 000	• Observational	• Pregnancy registry	• SSD: Nov. 2016 • DE: 2024

Japanese Encephalitis Vaccine
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT02933710	Phase 4 Postmarketing Surveillance Study for IMOJEV® in Republic of Korea	632	• Multi-center, open, observational, active safety surveillance study	• Occurrence of solicited and unsolicited events	• SSD: Jul. 2016 • DE: 2019

MenQuad TT Vaccine
Greater Europe Region

Study	Description	Patients	Design	Endpoints	Status
NCT03476135	Phase 3 3 Year Follow-up for Antibody Persistence & Booster in subjects previously vaccinated	188	• Open label, multicenter study to descibe immune persistence of the priming dose and immuno and safety of booster dose	• Immunogenicity and safety	• SSD: Feb. 2018 • DE: 2019