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APPENDICES
RESEARCH & DEVELOPMENT

February 7, 2018

R&D Pipeline – New Molecular Entities(*)

Phase 1 (Total:15)	Phase 2 (Total:15)	Phase 3 (Total:6)	Registration (Total:0)
SAR440340(**) Anti-IL33 mAb Asthma	**UshStat**® Myosin 7A gene therapy Usher Syndrome 1B	**SAR156597** IL4/IL13 bi-specific mAb Systemic Scleroderma	**SAR425899** GLP-1/GCG dual agonist Obesity/Overweight in T2D	**isatuximab** Anti-CD38 mAb Relapsing Refractory Multiple Myeloma (ICARIA)
SAR439794 TLR4 agonist Peanut Allergy	**SAR228810** Anti-protofibrillar AB mAb Alzheimer's Disease	**GZ389988** TRKA antagonist Osteoarthritis	**mavacamten**(8)(**) Myosin inhibitor Obstructive Hypertrophic Cardiomyopathy	**avalglucosidase alfa** Neo GAA Pompe Disease
SAR408701 Maytansin-loaded anti-CEACAM5 mAb Solid Tumors	**SAR442168**(3) BTK inhibitor Multiple Sclerosis	R **cemiplimab**(5)(**) PD-1 inhibitor mAb Advanced CSCC (Skin cancer)	**SAR407899** rho kinase Microvascular Angina	**fitusiran**(10) siRNA targeting Anti-Thrombin Hemophilia
SAR439459 anti-TGFβ mAb Advanced Solid Tumors	**SAR438335** GLP-1/GIP dual agonist Type 2 Diabetes	R **SAR566658** Maytansin-loaded anti-CA6 mAb Triple Negative Breast Cancer	Combination **ferroquine / OZ439**(**) Antimalarial	**sotagliflozin**(**) Oral SGLT-1&2 inhibitor Type 1 Diabetes
O **REGN3767**(1) Anti LAG-3 mAb Advanced Cancers	**SAR440181**(4)(**) Myosin activation Dilated Cardiomyopathy	R **olipudase alfa** rhASM Acid Sphingomyelinase Deficiency(6)	**Tuberculosis** Recombinant subunit vaccine	**SAR341402** Rapid acting insulin Type 1/2 Diabetes
SAR439859 SERD Metastatic Breast Cancer	**SAR247799** S1P1 agonist Cardiovascular indication	O **SAR339375**(7) miRNA-21 Alport Syndrome	**HIV** Viral vector prime & rgp120 boost vaccine	**efpeglenatide**(**) Long-acting GLP-1 agonist Type 2 Diabetes
O **lumasiran**(2) Investigational RNAi therapeutic Primary Hyperoxaluria Type 1 (PH1)	**Herpes Simplex Virus Type 2** HSV-2 vaccine	**venglustat** Oral GCS inhibitor Gaucher related Parkinson's Disease	**SP0232**(9) **mAb**(**) Respiratory syncytial virus Monoclonal Antibody	
	Respiratory syncytial virus Infants Vaccines	**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 Disease
- MS, Neuro, Gene therapy
- Diabetes
- Cardiovascular & metabolism
- Infectious Diseases
- Vaccines

(1) Regeneron product for which Sanofi has opt-in right
(2) Alnylam product for which Sanofi has opt-in right
(3) Also known as PRN2246
(4) Also known as MYK491
(5) Also known as SAR439684 and REGN2810
(6) Also known as Niemann Pick type B
(7) Regulus product for which Sanofi has opt-in right
(8) Also known as SAR439152 and MYK461
(9) Also known as MEDI8897

(10) Clinical hold lifted by FDA announced on Dec 15, 2017 - Clinical trial dosing to resume in Q1 2018. Following the Alnylam/Sanofi strategic restructuring of the RNAi therapeutics rare disease alliance announced in January 2018, Sanofi will have global rights on fitusiran. The transaction is subject to customary closing conditions and clearances, including clearance under the Hart-Scott Rodino Antitrust Improvements Act.
(*) 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:10)		Phase 3 (Total:16)		Registration (Total:3)
isatuximab + cemiplimab(1)(**) Anti-CD38 mAb + PD-1 inhibitor mAb Relapsing Refractory Multiple Myeloma	**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 1st line Newly Diagnosed Multiple Myeloma (IMROZ)	**dupilumab**(**) Anti-IL4Rα mAb Asthma 12y+ U.S.
isatuximab Anti-CD38 mAb + CyBord(2) Newly Diagnosed Multiple Myeloma	**sarilumab**(**) Anti-IL6R mAb Polyarticular Juvenile Idiopathic Arthritis	**Rabies VRVg** Purified vero rabies vaccine	**dupilumab**(**) Anti-IL4Rα mAb Nasal Polyposis	**isatuximab** Anti-CD38 mAb Relapsing Refractory Multiple Myeloma (IKEMA)	**VaxiGrip® QIV IM** Quadrivalent inactivated Influenza vaccine (6 - 35 months)
SAR439459 + cemiplimab(1)(**) Anti-TGFβ mAb + PD1 inhibitor mAb Advanced Solid Tumors	**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.)
O **cemiplimab**(1)(**) **+ REGN3767**(3) PD-1 inhibitor mAb + anti LAG-3 mAb Advanced Cancers	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	
SAR439859 SERD + Palbociclib Metastatic Breast Cancer	**venglustat** Oral GCS inhibitor Gaucher Disease Type 3		**Dupixent®**(**) Anti-IL4Rα mAb Atopic Dermatitis 6 months - 5 years old	**Praluent®**(**) Anti-PCSK9 mAb CV events reduction	
	venglustat Oral GCS inhibitor Fabry Disease		**cemiplimab**(1)(**) PD-1 inhibitor mAb 2nd line Cervical Cancer	**Fluzone® QIV HD** Quadrivalent inactivated Influenza vaccine - High dose	
			cemiplimab(1)(**) PD-1 inhibitor mAb 1st line NSCLC	**Men Quad TT** Advanced generation meningococcal ACYW conjugate vaccine	
			sotagliflozin(**) Oral SGLT-1&2 inhibitor Type 2 Diabetes	**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 Disease
- MS, Neuro, Gene therapy
- 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 - included in totals

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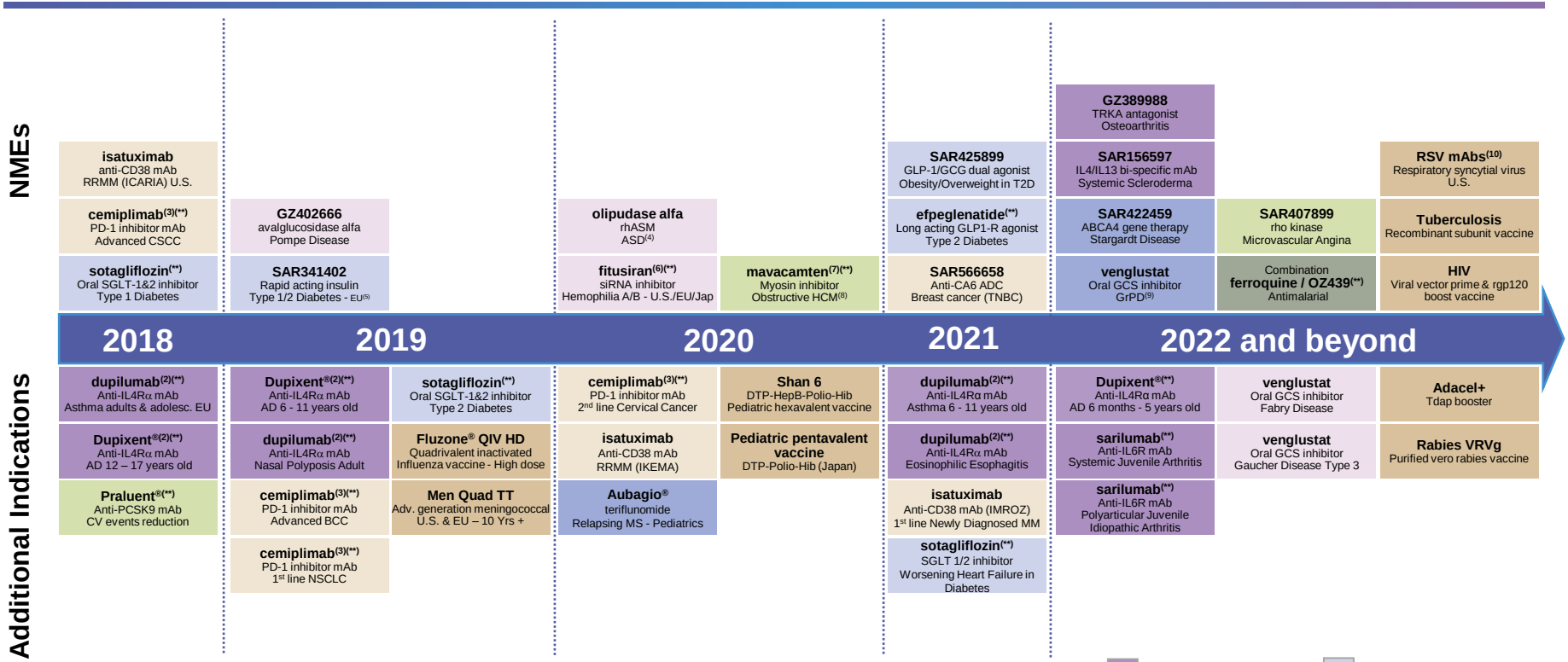
Expected Submission Timeline(1)



(1) Excluding Phase 1 - Data related to all studies published on clinicaltrials.gov
(2) Also known as SAR231893
(3) Also known as SAR439684 and REGN2810
(4) Acid Sphingomyelinase Deficiency
(5) Submission strategy for the U.S. under evaluation
(6) Clinical hold lifted by FDA announced on Dec 15, 2017 - Clinical trial dosing to resume in Q1 2018. Following theAlnylam/Sanofi strategic restructuring of the RNAi therapeutics rare disease alliance announced in January 2018, Sanofi will have global rights on fitusiran. The transaction is subject to customary closing conditions and clearances, including clearance under the Hart-Scott Rodino Antitrust Improvements Act.
(7) Also known as SAR439152 and as MYK461
(8) Hypertrophic Cardiomyopathy
(9) Gaucher Related Parkinson's Disease
(10) Also known as SP0232 and MEDI8897
(**) Partnered and/or in collaboration – Sanofi may have limited or shared rights on some of these products

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

Pipeline Movements Since Q3 2017

	Additions to the pipeline		Removals from the pipeline	
Phase 1	**SAR439859** SERD Metastatic Breast Cancer	**SAR442168**[1] BTK inhibitor Multiple Sclerosis	**SAR428926** Maytansin-loaded anti-Lamp1 mAb Cancer	**GZ402668** GLD52 (anti-CD52 mAb) Relapsing Multiple Sclerosis
Phase 2	**SAR407899** rho kinase Microvascular Angina		**SAR100842** LPA1 receptor antagonist Systemic Sclerosis **SAR156597** IL4/IL13 bi-specific mAb Idiopathic Pulmonary Fibrosis	**isatuximab** Anti-CD38 mAb monotherapy Acute Lymphoblastic Leukemia
Phase 3	**SAR341402** Rapid acting insulin Type 2 Diabetes **cemiplimab**[2](**) PD-1 inhibitor mAb 2nd line Cervical Cancer	**efpeglenatide**(**) Long-acting GLP-1 receptor agonist Type 2 Diabetes **isatuximab** Anti-CD38 1st line Newly Diagnosed Multiple Myeloma (IMROZ)	**Clostridium difficile** Toxoid vaccine	
Registration	**dupilumab**(**) Anti-IL4Rα mAb Asthma 12y+ U.S.		**patisiran**[3] siRNA inhibitor targeting TTR Hereditary ATTR Amyloidosis	

(1) Also known as PRN2246
(2) Also known as SAR439684 and REGN2810
(3) Following the Alnylam/Sanofi strategic restructuring of the RNAi therapeutics rare disease alliance announced in January 2018, Alnylam will have global rights on patisiran and Sanofi will receive royalties based on net sales of patisiran. The transaction is subject to customary closing conditions and clearances, including clearance under the Hart-Scott Rodino Antitrust Improvements Act".
(4) (**) 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	2	5	5	1	13
Oncology	9	3	5	0	17
Rare Diseases	1	4	2	0	7
Multiple Sclerosis, Neurology, Gene therapy	3	2	2	0	7
Diabetes	1	2	4	0	7
Cardiovascular Diseases	2	2	1	0	5
Infectious Diseases	0	1	0	0	1
Vaccines	2	6	3	2	13
TOTAL	**20**	**25**	**22**	**3**	

45 **25**

70 **Total Projects**

(1) Includes 3 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	Pharmacodynamic
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(1)
Asthma 1/3

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

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	2,287 expected	• 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

(1) Anti-IL4Rα mAb

Dupilumab(1)
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

(1) Anti-IL4Rα mAb

Dupilumab[1]
Asthma 3/3

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

(1) Anti-IL4Rα mAb

Dupilumab[(1)]
Atopic Dermatitis (AD)

Study	Description	Patients	Design	Endpoints	Status
AD R668-AD-1225 NCT01949311	Phase 3 Open-label Study of Dupilumab in Patients With Atopic Dermatitis	2000	• For patients who participated in placebo-controlled dupilumab atopic dermatitis (AD) trials • Open label extension study, Single-group	• Primary: Incidence and rate of TEAEs • Secondary: SAEs and AEs of special interest, % of patients who achieve and maintain a score of 0 to 1 on Investigator's Global Assessment (IGA), % of patients achieving and maintaining at least 75% reduction in EASI score over time, % of patients with IGA ≤ 2 at each visit, Change and % of change in EASI (50/75/90) and IGA, % of patients with reduction of Pruritus (NRS), Time to first remission, Time to relapse, % of patients requiring rescue therapy, QoL, Patient Oriented Eczema Measure (POEM),	• SSD: Oct. 2013 • DE: 2018

(1) Anti-IL4Rα mAb

Dupilumab(1)
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: 2018
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

(1) Anti-IL4Rα mAb

Dupilumab(1)
Atopic Dermatitis (AD)

Study	Description	Patients	Design	Endpoints	Status
LIBERTY AD PRESCHOOL 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 QoL Index	• SSD: Dec. 2017 • DE: 2022
AD in 6 - 11 Years Old 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

(1) Anti-IL4Rα mAb

Dupilumab(1)
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

(1) Anti-IL4Rα mAb

Sarilumab(1)
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

(1) Anti-IL6 mAb

Sarilumab[1]
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, The 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)[2]: 2018

(1) Anti-IL6 mAb
(2) Final Data Collection date for primary outcome measure

SAR156597[(1)]
Scleroderma

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

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)[(2)]: 2018

(1) Anti-IL13/IL4 mAb
(2) Final Data Collection date for primary outcome measure

SAR440340(1)
Asthma

Study	Description	Patients	Design	Endpoints	Status
Asthma NCT02999711	Phase 1 Assess the safety and tolerability of multiple ascending subcutaneous doses of REGN3500 in adult patients with Moderate Asthma	24	• Randomized, double-blind, Placebo-controlled, Multiple ascending dose study of the safety	• 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: Jan. 2017 • DE: Nov. 2018

(1) Anti-IL33 mAb

SAR440340(1)
Asthma – Combination with dupilumab

Study	Description	Patients	Design	Endpoints	Status
Asthma ACT15102 NCT03387852	Phase 2A Efficacy, Safety and Tolerability of SAR440340 alone and as Combination Therapy With Dupilumab in Moderate-to-Severe Asthma Patients 12-week Proof-of-Concept (PoC) Study	240	• Adult patients with Moderate-to-severe Asthma for at least 12 months, who are not well controlled on inhaled corticosteroid (ICS) Plus Long-acting β2 Adrenergic Agonist (LABA) Therapy • Randomized, Double-blind, Placebo-controlled, Parallel-group (SAR440340, dupilumab, SAR440340+dupilumab, placebo) on top of Fluticasone or Fluticasone/salmeterol combination • Study duration: approximately 36 weeks, including 4 weeks screening, 12 weeks treatment, and 20 weeks post-treatment.	• Primary: % of patients with Loss of Asthma Control (LOAC) • Secondary: Change in FEV1 (Forced Expiratory Volume in 1 second)	• SSD: March 2018 • DE: 2019

(1) Anti-IL33 mAb

Cemiplimab(1)
Advanced Malignancies (AM)

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

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	1,167	• 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

(1) PD-1 inhibitor

Cemiplimab(1)
Advanced Malignancies (AM)

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

Study	Description	Patients	Design	Endpoints	Status
PK in Japanese patients AM R2810-ONC-1622 NCT03233139	Phase 1 To investigate the safety and PKs of cemiplimab in Japanese patients with AM	6	• 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

(1) PD-1 inhibitor

Cemiplimab(1)
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)(2): 2018

(1) PD-1 inhibitor
(2) Final Data Collection date for primary outcome measure

Cemiplimab(1)
Cutaneous Squamous Cell Carcinoma (CSCC)

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

Study	Description	Patients	Design	Endpoints	Status
Advanced CSCC R2810-ONC-1540 NCT02760498	Phase 2 Cemiplimab monotherapy for patients with metastatic (nodal or distant) CSCC (Groups 1 and 3) or with unresectable locally advanced CSCC (Group 2)	150	• 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

(1) PD-1 inhibitor

Cemiplimab(1)
Basal Cell Carcinoma (BCC)

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

Study	Description	Patients	Design	Endpoints	Status
BCC R2810-ONC-1620 NCT03132636	Phase 2 Cemiplimab in patients with Advanced BCC who experienced progression of disease on Hedgehog Pathway Inhibitor Therapy, or were intolerant of Prior Hedgehog Pathway Inhibitor Therapy	147	• 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) (2): 2018

(1) PD-1 inhibitor
(2) Final Data Collection date for primary outcome measure

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

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

(1) PD-1 inhibitor

Cemiplimab[1]
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	800	• 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)[2]: 2020

(1) PD-1 inhibitor
(2) Final Data Collection date for primary outcome measure

Isatuximab(1)
Hematological Malignancies (HM)

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

Study	Description	Patients	Design	Endpoints	Status
CD38+HM TED10893 NCT01084252	Phase1/2 Dose escalation and efficacy study of isatuximab in patients with selected CD38+ HM	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

(1) Anti-CD38 mAb

Isatuximab(1)
Multiple Myeloma (MM)

Study	Description	Patients	Design	Endpoints	Status
Lenalidomide Combination **RRMM** TCD11833 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

(1) Anti-CD38 mAb

Isatuximab(1)
Multiple Myeloma (MM)

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

Study	Description	Patients	Design	Endpoints	Status
Pomalidomide Combination **RRMM** TCD14079 NCT02283775	Phase 1b Isatuximab, in combination with pomalidomide and dexamethasone for the Tx of Relapsed/Refractory MM	45	• 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 (escalating doses) + pomalidomide + dexamethasone • Total duration for one patient: up to 21 days screening, Tx period up to disease progression or AEs , 60- day follow-up	• Primary: DLTs, N of patients with AE • Secondary: ORR, PK, Immunogenicity, DOR, CB	• SSD: May 2015 • DE: 2018

(1) Anti-CD38 mAb

Isatuximab(1)
Multiple Myeloma (MM)

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

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: 2024

(1) Anti-CD38 mAb

Isatuximab(1)
Multiple Myeloma (MM)

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

Study	Description	Patients	Design	Endpoints	Status
RRMM TED14154 NCT02514668	Phase 1 Safety, PK and Efficacy of isatuximab in patients with Relapsed/Refractory MM	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: 2019

(1) Anti-CD38 mAb

Isatuximab(1)
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

(1) Anti-CD38 mAb

Isatuximab(1)
Multiple Myeloma (MM)

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

Study	Description	Patients	Design	Endpoints	Status
Cemiplimab Combination **RRMM** TCD14906 NCT03194867	Phase 1 Phase 2 Safety, PK and Efficacy of isatuximab in combination with cemiplimab in patients with Relapsed/Refractory MM	54	• 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 • Open-label, Single Group assignment • Isatuximab + cemiplimab • 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	• Primary: DLTs, N of patients with AE, ORR • Secondary: CB, DOR, TTR, PFS, OS, PK, Immunogenicity (isatuximab and cemiplimab)	• Not yet recruiting

(1) Anti-CD38 mAb

Isatuximab[1]
Multiple Myeloma (MM)

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

Study	Description	Patients	Design	Endpoints	Status
ICARIA-MM RRMM EFC14335 NCT02990338	Phase 3 Isatuximab, pomalidomide, and dexamethasone to pomalidomide and dexamethasone in Refractory or Relapsed and RRMM	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)[2]: 2018

(1) Anti-CD38 mAb
(2) Final Data Collection date for primary outcome measure

Isatuximab(1)
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)(2): 2020

(1) Anti-CD38 mAb
(2) Final Data Collection date for primary outcome measure

Isatuximab(1)
Multiple Myeloma (MM)

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

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) (2): 2022

(1) Anti-CD38 mAb
(2) Final Data Collection date for primary outcome measure

SAR566658[(1)]
Triple Negative Breast Cancer (TNBC)

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

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

(1) Maytansin loaded anti-CA6 mAb

SAR439459(1)
Advanced Solid Tumors (AST)

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

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

(1) TGFß inhibitor mAb

SAR408701(1)
Advanced Solid Tumors (AST) 1/2

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

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	152 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	• Primary: MTD, Anti-tumor response RECIST • Secondary: Safety, Immunogenicity, PK	• SSD: Sep. 2014 • DE: 2019

(1) Maytansin loaded anti-CEACAM5 mAb

SAR408701(1)
Advanced Solid Tumors (AST) 2/2

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

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: 2019

(1) Maytansin loaded anti-CEACAM5 mAb

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

Avalglucosidase alfa
Pompe disease (PD) 2/3

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

Study	Description	Patients	Design	Endpoints	Status
Mini-COMET Infantile Onset ACT14132 NCT03019406	Phase 2 To assess safety and efficacy of avalglucosidase alfa 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

Avalglucosidase alfa
Pompe disease (PD) 3/3

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

Study	Description	Patients	Design	Endpoints	Status
NEO-EXT LTS13769 NCT02032524	Phase 2 Phase 3 Long-term safety and PK of repeated biweekly infusions of avalglucosidase alfa 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)(2)]
Hereditary ATTR (hATTR) Amyloidosis

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

Study	Description	Patients	Design	Endpoints	Status
APOLLO Global OLE FAP LTE14730 ALN-TTR02-006 NCT02510261	Phase 3 Study of Patisiran for the Tretament of patients with hereditary transthyretin mediated amyloidosis (hATTR)	228	• For patients having completed a previous patisiran efficacy study • Safety and tolerability of long-term dosing of patisiran • Single Group assignment, 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 will have global rights on patisiran and Sanofi will receive royalties based on net sales of patisiran. The transaction is subject to customary closing conditions and clearances, including clearance under the Hart-Scott Rodino Antitrust Improvements Act".

(2) SiRNA targeting TTR

Fitusiran[1][2]
Hemophilia A & B

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: 2019

(1) Clinical hold lifted by FDA announced on Dec 15, 2017 - Clinical trial dosing to resume in Q1 2018. Following the Alnylam/Sanofi strategic restructuring of the RNAi therapeutics rare disease alliance announced in January 2018, Sanofi will have global rights on fitusiran. The transaction is subject to customary closing conditions and clearances, including clearance under the Hart-Scott Rodino Antitrust Improvements Act.
(2) SiRNA targeting Antithrombin/AT3

Olipudase Alfa[(1)] 1/3
Acid Sphingomyelinase Deficiency (ASMD)

Study	Description	Patients	Design	Endpoints	Status
ASCEND **Niemann-Pick disease type B[(2)]** 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 • 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)[(3)]: 2019

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

Olipudase Alfa[(1)] 2/3
Acid Sphingomyelinase Deficiency (ASMD)

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

Study	Description	Patients	Design	Endpoints	Status
ASCEND Peds DFI13803 NCT02292654	Phase 1 Phase 2 Safety, Tolerability, PK, and efficacy evaluation of ollipudase 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: N of AE, 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

(1) RhASM ERT

Olipudase Alfa[(1)] 3/3
Acid Sphingomyelinase Deficiency (ASMD)

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

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: N of patients experiencing AE, Physical examinations including neurologic examinations, Clinical laboratory tests, Safety biomarkers, IR assessments, 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: 2021

(1) RhASM ERT

Venglustat(1)
Fabry disease (FD)

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

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

(1) GCS inhibitor

Venglustat[1]
Gaucher disease (GD) Type 3

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

Study	Description	Patients	Design	Endpoints	Status
LEAP GD Type 3 PDY13949 NCT02843035	Phase 2 Tolerability, PK, PD, and exploratory efficacy of venglustat in combination with cerezyme in adult patients with GD Type 3	10	• 52-week Two-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 Tx with venglustat in Part 2, Total duration 45 days • Part 2 : Safety and tolerability in GD3 patients, Total duration up to 61 weeks including 52 weeks of treatment	• Primary: N of patients with AE, Change from baseline in biomarker levels (CSF and Plasma) • Secondary: PK parameters (CSF and Plasma)	• SSD: Mar. 2017 • DE (1st Part)[2]: 2021

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

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)

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

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 With Disease Activity on Prior DMT	50	• Patients with Relapsing Remitting Multiple Sclerosis (RRMS), according to McDonald 2010 and International Pediatric Multiple Sclerosis Study Group criteria, aged from 10 years to less than 18 years at study entry • At least 2 recorded MS attacks, and at least 1 MS attack (relapse) in the last year during treatment with an IFNB or GA after having been on that therapy for at least 6 months • Open-label, rater-blinded, single-arm, before and after switch study of efficacy, safety and tolerability of alemtuzumab	• Primary: Efficacy, Safety, and Tolerability • Secondary: PK, PD, Anti-drug antibody formation, and potential effects of alemtuzumab on other MS disease characteristics such as cognition and QoL	• SSD: November 2017 • DE: March 2025

SAR422459(1)
Stargardt Disease

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

Study	Description	Patients	Design	Endpoints	Status
Stargardt's Macular Degeneration TDU13583 NCT01367444	Phase 1 Phase 2a Safety and tolerability of ascending doses of SAR422459 in patients with Stargardt's Macular Degeneration	46	• Patients with a diagnosis of Stargardt's Macular Degeneration, with at least one pathogenic mutant ABCA4 allele on each chromosome • Non-randomized, Single Group assignment, Open-label, ascending doses	• Primary: IAE, Change from baseline in ocular safety assessments • Secondary: Delay in retinal degeneration	• SSD: Jun. 2011 • DE: 2020
Stargardt's Macular Degeneration LTS13588 SG1/002/11 NCT01736592	Phase 2b Long term safety, tolerability and Biological activity of an experimental gene transfer agent, SAR422459, designed to treat patients With Stargardt Macular Degeneration	28	• Long Term follow up of patients who received SAR422459 in a previous study (TDU13583) • Single Group assignment, Open-label • Follow-up 15 years	• Primary: IAE • Secondary: Delay in retinal degeneration	• SSD: 2012 • DE: 2036

(1) ABCA4 gene therapy

SAR421869(1)
Usher 1B Syndrome

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

Study	Description	Patients	Design	Endpoints	Status
UshStat® **Usher Syndrome Type 1B** TDU13600 NCT01505062	Phase 1 Phase 2a Safety and tolerability of ascending doses of subretinal injections of UshStat® in patients with Retinitis Pigmentosa associated with Usher syndrome Type 1B	18	• Patients with clinical and molecular diagnosis of Retinitis Pigmentosa associated with Usher Syndrome type 1B. With at least one pathogenic mutation in the MYO7A gene on each chromosome • Non-randomized, Single Group assignment, Open-label, ascending doses	• Primary: IAE • Secondary: Delay in retinal degeneration	• SSD: 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) • Single Group assignment, Open-label	• Primary: IAE • Secondary: Change from baseline in ocular safety assessments, Delay in retinal degeneration	• SSD: Dec. 2012 • DE: 2035

(1) Myosin 7A gene therapy

GZ402668(1)
Relapsing Multiple Sclerosis (RMS)

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

Study	Description	Patients	Design	Endpoints	Status
Long Term Follow-Up **MS** LTS14120 NCT02313285	Phase 2b Open-label, Long-term follow-up study of MS patients who participated in previous Genzyme-sponsored studies of GZ402668	72	• Long Term safety follow up of patients who received GZ402668 in a previous study (TDU13475 or TDU14981) • No administration of GZ402668 in the LTS14120 study, Patients who already received investigational medicinal product (GZ402668 or placebo) in TDU13475 or TDU14981 will be followed up to 47 months in the LTS14120	• Primary: N of patients with AE, Safety, as assessed by clinical (physical examination), laboratory (hematology, creatinine, and urinalysis with microscopy), ECG, vital sign events, Clinically significant changes in thyroid function tests from baseline • Secondary: Time to lymphocyte repopulation, N of patients with anti-drug antibodies	• SSD: Jan. 2015 • DE: 2022

(1) Anti-CD52 mAb

Venglustat[1]
GBA-PD

Study	Description	Patients	Design	Endpoints	Status
MOVES-PD ACT14820 NCT02906020	Phase 2 Drug Dynamics, Efficacy, Safety of venglustat in Parkinson's Disease (PD) patients carrying a Glucocerebrosidase (GBA) Gene Mutation	27 patients / Part 1 216 patients / Part 2	• Male and female adults with a diagnosis of PD and who are heterozygous carriers of a GBA mutation associated with PD • 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

(1) GCS inhibitor

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)(1)

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

(1) Japan

Insulin glargine / lixisenatide
Type 2 Diabetes Mellitus (T2DM)(1)

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

(1) Japan

Insulin glargine / lixisenatide
Type 2 Diabetes Mellitus (T2DM)(1)

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

(1) Japan

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(1)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-MONO (301) **T2DM** **EFC14833** NCT02926937	Phase 3 Efficacy and safety of sota (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 sota dose 1 vs. placebo • Secondary: Change from baseline in 2-hour PPG following a mixed meal in comparison of sota doses 1/2 vs. placebo, FPG in comparison of sota dose 1 vs. placebo, Body weight in comparison of sota doses 1/2 versus placebo, % of patients with HbA1c <6.5% in comparison of sota dose 1 vs. placebo, % of patients with HbA1c <7.0% in comparison of sota dose 1 vs. placebo, Change from Baseline in HbA1c in comparison of sota dose 2 vs. placebo, Change from baseline in SBP for patients with baseline SBP ≥130 mmHg in comparison of sota dose 1 vs. placebo and SBP for all patients in comparison of sota doses 1/2 vs. placebo	• SSD: Dec. 2016 • DE: 2019

(1) SGLT 1/2 inhibitor

Sotagliflozin(1)
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

(1) SGLT 1/2 inhibitor

Sotagliflozin(1)
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

(1) SGLT 1/2 inhibitor

Sotagliflozin(1)
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

(1) SGLT 1/2 inhibitor

Sotagliflozin(1)
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

(1) SGLT 1/2 inhibitor

Sotagliflozin(1)
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

(1) SGLT 1/2 inhibitor

Sotagliflozin(1)
Type 2 Diabetes Mellitus (T2DM)

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

(1) SGLT 1/2 inhibitor

Sotagliflozin(1)
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

(1) SGLT 1/2 inhibitor

Sotagliflozin(1)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-EMPA T2DM **EFC14867** NCT03351478	Phase 3 Efficacy and Safety of Sotagliflozin Versus Placebo and Empagliflozin in Subjects With Type 2 Diabetes Mellitus Who Have Inadequate Glycemic Control While Taking a DPP4 Inhibitor Alone or With Metformin	700	• Patients with Type 2 Diabetes on Dipeptidyl peptidase-4 inhibitors(DPP4(i)) with or without metformin at a stable dose for at least 12 weeks prior to Screening Visit • Randomized, Double-blind, Controlled, Parallel-group (sotagliflozin, empagliflozin, placebo) • Total Study duration: Up to 34 weeks, including a Screening Phase of up to 2 weeks, a 2 week Run-In Phase, a 26-week double-blind Treatment Period and a 4-week post-treatment Follow-up	• Primary: Absolute change in hemoglobin A1c (HbA1c) • Secondary: Change in sitting SBP in patients with SBP≥ 130 mmHg at baseline, Change in 2-hour Post Prandial Glucose, Change in Fasting Plasma Glucose, Change in Body Weight, Change in sitting SBP for all patients, % of patients with Hb1Ac < 6,5% and < 7% at week 26	• SSD: Nov. 2017 • DE: 2019

(1) SGLT 1/2 inhibitor

Sotagliflozin(1)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SOTA-BONE T2DM **EFC15294** NCT03386344	Phase 3 Efficacy and Bone Safety of Sotagliflozin Dose 1 and Dose 2 Versus Placebo in Subjects With Type 2 Diabetes Mellitus Who Have Inadequate Glycemic Control	360	• Randomized, Double-blind, Placebo-controlled, Parallel-group (sota dose 1, sota dose 2, placebo), on top of baseline antidiabetic therapy • Total study duration approximately 110 weeks: Screening period of up to 2 weeks, 2 week single-blind run-in period, a 26-week double-blind core treatment period, a 78-week double-blind extension period, and a 2- week post treatment follow up period	• Primary: Change in hemoglobin A1c (HbA1c) (for sotagliflozin dose 1) • Secondary: Change in HbA1c for sota dose 2 compared to placebo, Change in Body Weight, Change in Fasting Plasma Glucose, Change in SBP, % of patients with HbA1c<7% at week 26, Change in bone mineral density (BMD) of lumbar spine, Change in BMD of total hip, Change in BMD of femoral neck, adverse events	• SSD: Jan. 2018 • DE: 2020

(1) SGLT 1/2 inhibitor

Efpeglenatide(1)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
T2DM **EFC14822** NCT03353350	Phase 3 Efficacy and Safety of Efpeglenatide Versus Placebo in Patients With Type 2 Diabetes Mellitus Inadequately Controlled With Diet and Exercise	400	• Patients with T2DM, with HbA1c between 7 and 10 % at screening and treated with diet and exercise, • Randomized, Double-blind, Placebo-controlled, Parallel-group (efpe low dose, efpe middle dose, efpe high dose, placebo) • Total study duration: approximately 65 weeks, including a 3-week screening period, 30 weeks core treatment period, 26 weeks extension treatment period, and 6 weeks safety follow up.	• Primary: Change in HbA1c • Secondary: Change in Fasting Plasma Glucose,Change in self-monitored Plasma Glucose, % of patients with HbA1c < 7% at week 30 and week 56, % of patients starting rescue therapy during ttmt period, Time to rescue, Change in Body Weight,	• SSD: Dec. 2017 • DE: 2020

(1) Long-acting GLP-1 agonist

SAR341402(1)
T1 & T2 DM

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

(1) Rapid Acting Insulin

SAR425899(1)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SAR425899 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

(1) GLP-1R/GCGR

SAR425899(1)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SAR425899 T2DM **PDY15012** NCT03376802	Phase 1 Effect of Repeated Subcutaneous Doses of SAR425899 on Energy Expenditure and Safety in Overweight to Obese subjects	30	• Overweight to obese male and female subjects with a body mass index 28 – 35 kg/m2. • Randomized, Double-blind, Placebo-controlled, Parallel groups (SAR425899, placebo), • Study duration: approximately 7-8 weeks: screening period, 19-day treatment period and end of study visit 7 days after last dosing	• Primary: change of sleep energy expenditure • Secondary: change of Total energy expenditure, Resting energy expenditure, Basal energy expenditure, Respiratory quotient, Fat mass and fat-free mass, Diet induced thermogenesis, Fasting plasma glucose, HbA1c, lipids biomarkers, ketones, PK parameters	• SSD: Jan. 2018 • DE: 2018

(1) GLP-1R/GCGR

SAR425899(1)
Type 2 Diabetes Mellitus (T2DM)

Study	Description	Patients	Design	Endpoints	Status
SAR425899 T2DM **PDY15264** NCT03350191	Phase 1 To Investigate if SAR425899 Binds to the Liver and Pancreas in Overweight to Obese Type 2 Diabetes Mellitus Patients	14	• Patients with T2DM for at least 1 year at the time of inclusion with stable metformin treatment prior to inclusion, with or without comorbidities related to type 2 diabetes mellitus • With a body weight between 60.0 and 120.0 kg, inclusive, body mass index between 28.0 and 38.0 kg/m2, inclusive. • Non-Randomized, Open-label, Parallel groups (two SAR425899 doses), • Study duration: approximately 7 weeks with 20 days treatment period	• Primary: glucagon receptor occupancy in the liver • Secondary: GLP-1 receptor occupancy, change in fasting plasma glucose, in ketone bodies, in lipid biomarkers, in volume of distribution in the liver and in the pancreas, average standard uptake values of PET tracers in the liver and in the pancreas, PK parameters	• SSD: Dec. 2017 • DE: 2018

(1) GLP-1R/GCGR

Alirocumab(1)
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)	18 600	• 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

(1) anti-PCSK-9 mAb

Alirocumab(1)
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

(1) anti-PCSK-9 mAb

Alirocumab(1)
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(1)
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

(1) anti-PCSK-9 mAb

Alirocumab(1)
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

(1) anti-PCSK-9 mAb

Alirocumab(1)
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

(1) anti-PCSK-9 mAb

SAR439152(1)
Obstructive Hypertrophic Cardiomyopathy (OHCM)

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

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

(1) Myosin inhibitor

SAR407899(1)
Microvascular Angina (MA)

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

(1) Rho.kinase inhibitor

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

Adacel+
North America Region

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

Study	Description	Patients	Design	Endpoints	Status
NCT02587520	Phase 1 Study of Tetanus Toxoid, Reduced Diphtheria Toxoid, and Acellular Pertussis Vaccine Adsorbed in Healthy Subjects	1365	• 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
Co-administration w/ Tdap booster(1)

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

(1) Asia Pacific Region

Dengue Vaccine
Different schedules(1)

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

(1) Asia Pacific, Latin America Regions

Dengue Vaccine
Booster dose(1)

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	252	• Multi-center, observer-blind, randomized, placebo-controlled, Phase II trial	• Immunogenicity and safety of a booster dose	• SSD: Apr. 2016 • DE: 2019

(1) Latin America Region

Dengue Vaccine Booster(1)

Study	Description	Patients	Design	Endpoints	Status
NCT02824198	Phase 2 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

(1) Asia Pacific Region

Dengue Vaccine
Co-administration w/ HPV(1)

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

(1) Latin America Region

Dengue Vaccine
Co-administration w/ HPV(1)

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

(1) Asia Pacific Region

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: 2019

Flu Vaccine
Fluzone HD-QIV HV(1)

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,	• SSD: Sep. 2017 • DE: 2018

(1) North America Region

Flu Vaccine
Fluzone HD-QIV HV(1)

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: Oct. 2017 • DE: 2018

(1) Japan

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	1 600	• 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

Meninge Vaccine
MenQuadTT(1)

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: 2020

(1) Europe, Latin America, Asia Pacific Regions

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
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

Rabies Vaccine
Purified Vero Rabies(1)

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

(1) North America Region

Rabies Vaccine
Verorab(1)

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

(1) Asia Pacific Region