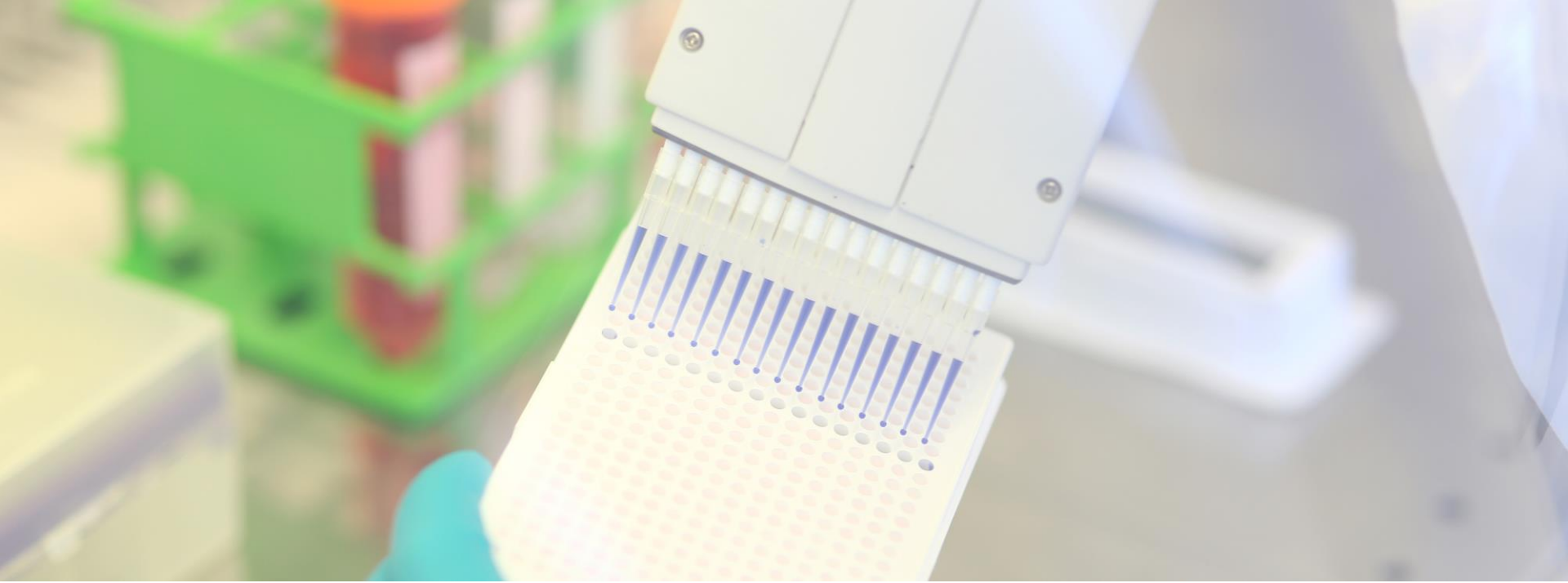

SANOFI

Clinical Trials Appendices

List of abbreviations

AE	Adverse Events
APO	Apolipoprotein
BOR	Best Overall Response
BW	Body Weight
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
DPP4	Dipeptidyl peptidase 4
EASI	Eczema Area and Severity Index
FPC	Fasting Plasma Glucose
HbA1c	Hemoglobin A1c
IAE	Incidence of Adverse Events
IAR	Infusion Associated Reaction
IC	Investigator's Choice
IGA	Investigator's Global Assessment
IMID	Immunomodulatory Drug
ITT	Intent To Treat
LP	Lipoprotein
MRI	Magnetic Resonance Imaging
MTD	Maximum Tolerated Dose
N	Number
NC	Nasal Congestion/obstruction
NNT	Number Needed to Treat
OS	Overall Survival
ORR	Overall Response Rate
PD	Pharmacodynamics
PI	Proteasome Inhibitor
PFS	Progression-Free Survival
PK	Pharmacokinetic
PPG	Postprandial Glucose
PRO	Patient Reported Outcome
QNW	Every N Weeks
QNM	Every N Months
QOL	Quality Of Life
RECIST	Response Evaluation Criteria in Solid Tumors
SAE	Serious Adverse Events
SBP	Systolic Blood Pressure
SDMT	Symbol Digit Modalities Test
SMPG	Self Monitored Plasma Glucose
SSD	Study Start Date
TC	Total Cholesterol
TEAE	Treatment Emergent Adverse Events
TSS	Total Symptom Score
TG	Triglycerides
TTP	Time To Progression
TTR	Time To Response
TX	Treatment
VGPR	Very Good Partial Response

Dupilumab (anti-IL4Rα mAb)
Asthma (1/3)

Study	Description	Patients	Design	Endpoints	Status
Continuation of LIBERTY ASTHMA TRAVERSE LPS15023 NCT03620747	Phase 3 Continuation of TRAVERSE evaluating Dupilumab safety in Patients with Asthma (Long term follow-up)	750	• Patients with asthma who completed the treatment period in the previous dupilumab asthma clinical study LTS12551 • Open-label, Single group assignement	• Primary: TEAEs: % of patients reporting TEAs, event rates per 100 patient-year	• SSD: Aug. 2018 • DE: 2022
LIBERTY ASTHMA EXCURSION LTS14424 NCT03560466	Phase 3 Long term safety and tolerability (1 year) of dupilumab in pediatric patients with asthma who participated in a previous dupilumab asthma clinical study	354	• Open-label • 1 year of Tx	• Primary: N of patients experiencing any TEAE • Secondary: Severe asthma exacerbation events, change in % predicted FEV1, in absolute FEV1, in FVC, FEF, dupilumab concentrations, anti-dupilumab Ab, eosinophils, Ig, IgE	• SSD: June 2018 • DE: 2021

Dupilumab (anti-IL4Rα mAb)
Asthma (2/3)

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	408	• 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: 2020

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

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

Dupilumab (anti-IL4Rα mAb)
Atopic Dermatitis (AD) (1/4)

Study	Description	Patients	Design	Endpoints	Status
OLE Pediatrics AD R668-AD-Reg 1434 NCT02612454	Phase 3 A study to assess the long-term safety of dupilumab administered in patients 6 to <18 years of age with AD	765 expected	• For patients having participated in a prior dupilumab study in pediatrics with AD • Open label extension study	• Primary: Incidence and rate of TEAEs • Secondary: SAEs and TEAEs of special interest, % of patients who achieve and maintain remission, EASI-75: % of patients achieving and maintaining at least 75% reduction in EASI score over time, EASI-50: % of patients achieving and maintaining at least 50% reduction in EASI scores over time	• SSD: Oct. 2015 • DE: 2023

Dupilumab (anti-IL4Rα mAb)
Atopic Dermatitis (AD) (2/4)

Study	Description	Patients	Design	Endpoints	Status
LIBERTY AD PRESCHOOL R668-AD-1539 NTC03346434	Phase 2/3 Safety, Pharmacokinetics and Efficacy of Dupilumab in Patients ≥6 Months to <6 Years With Severe Atopic Dermatitis	280	• Part A: Open-label, single-ascending dose, sequential cohort phase 2 study • Part B: Randomized, double-blind, parallel-group, placebo-controlled phase 3 study	• Part A: PK • Part B: Proportion of patients with Investigator's Global Assessment "0" or "1" (on a 5-point scale) at week 16	• SSD: Dec. 2017 • DE: 2022

Dupilumab (anti-IL4Rα mAb)
Atopic Dermatitis (AD) (3/4)

Study	Description	Patients	Design	Endpoints	Status
Open-Label R668-AD-1225 NCT01949311	Phase 3 Open-Label study of Dupilumab in patients with Atopic Dermatitis	2678	• Open label extension study for patients who participated in placebo-controlled dupilumab AD trials. The study primarily evaluates long term safety (adverse events) and immunogenicity. Efficacy parameters are based on IGA, EASI) and the NRS.	• Primary: TEAEs, • Secondary: SAEs and AEs of special interest, % of patients who achieve and maintain remission, EASI-75: % of patients achieving and maintaining at least 75% reduction in EASI score over time, EASI-50: % of patients achieving and maintaining at least 50% reduction in EASI scores over time	• SSD: Oct. 2013 • DE: 2022

Dupilumab (anti-IL4Rα mAb)
Atopic Dermatitis (AD) (4/4)

Study	Description	Patients	Design	Endpoints	Status
AD in Chinese Patients EFC15116 NCT03912259	Phase 3 Efficacy and Safety of Dupilumab in Chinese Patients with Moderate-to-severe Atopic Dermatitis	163	• Chinese patients with chronic AD present for at elast 3 years before the screening visit, • Randomized, Double-Blind, Placebo-controlled • 2 Arms: dupilumab vs Placebo	• Primary: Investigator's Global Assessment (IGA), • Secondary:, % of patients with EASI-75 response, % of patients with reduction of peak daily pruritus NRS ≥ 4, % of patients with reduction of peak daily pruritus NRS ≥ 3, change in NRS, change in EASI score, change in BSA affected by AD, Dermatology QoL and EQ-5D, change in patients oriented eczema measure (POEM), sick-leave/missed school days proportion, AEs, dupimumab immunogenicity	• SSD: Dec 2018 • DE: 2020

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

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

Dupilumab (anti-IL4Rα mAb) adjunct to AR101
Peanut Allergy (1/2)

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

Dupilumab (anti-IL4Rα mAb)
Peanut Allergy (2/2)

Study	Description	Patients	Design	Endpoints	Status
Peanut Allergy R668 –ALG - 1702 NCT03793608	Phase 2 Efficacy and Safety of Dupilumab monotherapy in Pediatric Patients with Peanut Allergy	48	• Child 6 to 17 years experiencing dose-limiting symptoms at or before the challenge dose of peanut protein on screening: double-blind placebo-controlled food challenge (DBPCFC) and not experiencing dose-limiting symptoms to placebo • Randomized, double-blind, parallel assignment, placebo-controlled study, • 2 arms: dupilumab vs placebo	• Primary: % of patients who “pass” DBPCFC with low-dose (cumulative) peanut protein at week 24, • Secondary: % of patients that pass a DBPCFC with low-dose, mid-dose and high-dose of peanut protein, change in cumulative tolerated dose of peanut protein during DBPBFC, , % of change in peanut-specific IgE, change in titrated SPT.	• SSD: May 2019 • DE: 2022

Dupilumab (anti-IL4Rα mAb)
Chronic Obstructive Pulmonary Disease (COPD)

Study	Description	Patients	Design	Endpoints	Status
COPD BOREAS EFC15804 NCT03930732	Phase 3 Efficacy, Safety and Tolerability of Dupilumab in Patients with Moderate-to-Severe Chronic Obstructive Pulmonary Disease (COPD) with Type 2 inflammation	924	• Patients with COPD diagnosis, • Randomized, double-blind, parallel assignment, placebo-controlled study, • 2 arms: dupilumab vs placebo	• Primary: annual rate of acute COPD exacerbation, • Secondary: change in pre-bronchodilator FEV1, change in SGRQ score, Improvement in SGRQ, change in post-bronchodilator FEV1, change in forced expiratory flow (FEF), annualized rate of severe COPD exacerbations (AECOPD), time to first AECOPD, AEs, PCSA changes, dupilumab immunogenicity.	• SSD: May 2019 • DE: 2022

Dupilumab (anti-IL4Rα mAb)
Bullous Pemphigoid (BP)

Study	Description	Patients	Design	Endpoints	Status
LYBERTY-BP R668-BP-1902 NCT04206553	Phase 2/3 Efficacy, Safety of Dupilumab in Patients with Bullous Pemphigoid	80	• Patients with clinical features of BP, • Randomized, double-blind, parallel assignment, placebo-controlled study, • 2 arms: dupilumab vs placebo (+ oral cortocosteroides – OCS – in both arms)	• Primary: % of patients with sustained remission (off OCS), • Secondary: OCS-sparing effects, effect on itch, QoL, circulating BP180 and BP230 antibodies, safety and tolerability, Pharmacokinetic, immunogenicity.	• SSD: H1 2020 • DE: 2022

Dupilumab (anti-IL4Rα mAb)
Chronic Spontaneous Urticaria (CSU)

Study	Description	Patients	Design	Endpoints	Status
CUPID EFC16461 NCT04180488	Phase 3 Efficacy, of Dupilumab in adults and adolescents Patients with CSU who remain symptomatic despite the use of H1 antihistamine and who are naïve to Omalizumab (study A) And in adults and adolescents Patients with CSU who remain symptomatic despite the use of H1 antihistamine and who are Intolerant or Incomplete responders to Omalizumab (study B)	184	• Adults and adolescents (≥ 12 years) Patients with diagnosis of CSU refractory to H1 antihistamine, • Randomized, double-blind, parallel assignment, placebo-controlled study, • Studies A & B : 2 arms: dupilumab vs placebo (on top of sedating H1-antihistamine – OCS - in both arms)	• Primary: itch severity score (ISS7), • Secondary: urticaria activity score (UAS7), ISS7, hives severity score (HSS7), angioedema activity score (AAS7), urticaria control test (UCT), QoL, Patient Global Assessment, % of patients receiving OCS, safety.	• SSD: H1 2020 • DE: 2021

Dupilumab (anti-IL4Rα mAb)
Prurigo Nodularis (PN) (1/2)

Study	Description	Patients	Design	Endpoints	Status
PRIME EFC16459 NCT04183335	Phase 3 Efficacy and Safety of Dupilumab in Patients with PN Inadequately Controlled on Topical Prescription Therapies or When these Therapies are not Advisable	150	• Patients with a clinical diagnosis of PN, • Randomized, double-blind, parallel assignment, placebo-controlled study, • 2 arms: dupilumab vs placebo (on top of moisturezers and if applicable low to medium potent topical corticosteroids or topical calcineurin inhibitors, in both arms)	• Primary: improvement of worst-itch numeric rating scale (WI-NRS) ≥ 4 at week 12, • Secondary: % of patients with improvement in WI-NRS ≥ 4 at week 24, time to onset of effect on pruritus, change in WI-NRS, Investigator's global assessment on PN-Stage and PN-Assess, IGA PN-Score, QoL; safety, TEAs antidrug antibodies (ADA) against dupilumab.	• SSD: January 2020 • DE: 2021

Dupilumab (anti-IL4Rα mAb)
Prurigo Nodularis (PN) (2/2)

Study	Description	Patients	Design	Endpoints	Status
PRIME2 EFC16460 NCT04202679	Phase 3 Efficacy and Safety of Dupilumab in Patients with PN Inadequately Controlled on Topical Prescription Therapies or When these Therapies are not Advisable	150	• Patients with a clinical diagnosis of PN, • Randomized, double-blind, parallel assignment, placebo-controlled study, • 2 arms: dupilumab vs placebo (on top of moisturezers and if applicable low to medium potent topical corticosteroids or topical calcineurin inhibitors, in both arms)	• Primary: improvement of worst-itch numeric rating scale (WI-NRS) ≥ 4 at week 12, • Secondary: % of patients with improvement in WI-NRS ≥ 4 at week 24, time to onset of effect on pruritus, change in WI-NRS, Investigator's global assessment on PN-Stage and PN-Assess, IGA PN-Score, QoL; safety, TEAs antidrug antibodies (ADA) against dupilumab.	• SSD: January 2020 • DE: 2021

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

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

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

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

Study	Description	Patients	Design	Endpoints	Status
Polyarticular JIA Children & Adolescents SKYPP DRI13925 NCT02776735	Phase 2b Dose-finding study of sarilumab in children and adolescents with Polyarticular-course Juvenile Idiopathic Arthritis (pcJIA)	36 in core part, 60 total	• In children and adolescents, Aged 2 to 17 years, with pcJIA • Open-label, sequential, ascending, repeated dose-finding Study;	• Primary: PK parameters (Up to week 12) • Secondary: PD profile, The efficacy and the safety of sarilumab in patients with pcJIA, Long-term safety of sarilumab in patients with pcJIA	• SSD: Oct. 2016 • DE: 2018 (36 patients CSR); 2021 (60 patients CSR); 2023 (CSR with 3-year extension)
Systemic JIA Children & Adolescents SKYPS DRI13926 NCT02991469	Phase 2b Dose-finding study of sarilumab in children and adolescents with Systemic Juvenile Idiopathic Arthritis (sJIA)	24 in core part, 48 total	• In children and adolescents, aged 1 to 17 years, with sJIA • Open-label, sequential, ascending, repeated dose finding study, 4-week screening, 12-week coreTx, 144-week extension, 6-week post-Tx	• Primary: PK parameters (Up to week 12) • Secondary: PD profile, efficacy and the safety of sarilumab in patients with sJIA, Long term safety of sarilumab in patients with sJIA	• SSD: Sep. 2018 • DE: 2021 (24 patients CSR); 2023 (48 patients CSR), 2025 (CSR with 3-year extension)

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

Study	Description	Patients	Design	Endpoints	Status
GCA TAPERR EFC15068 NCT03600805	Phase 3 Efficacy of sarilumab in combination with corticosteroid in patients with Giant Cell Arteritis	360	• Patients suffering from GCA; new onset active disease or refractory active disease • Randomized, parallel assignment, double-blind, placebo controlled, 2 doses of sarilumab tested vs placebo, in association with prednisone • Study duration per patient: approximately 82 weeks: up to 6 weeks screening, 52-week Tx period, 26-week follow-up period	• Primary: % of patients achieving sustained remission at week 52 • Secondary: components of sustained remission, cumulative corticosteroid dose, time to 1st GCA flare, change in glucocorticoid toxicity index, AEs, PK,	• SSD: Nov. 2018 • DE: primary completion:2022, full completion 2023

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

Study	Description	Patients	Design	Endpoints	Status
PMR SAPHYR EFC15160 NCT03600818	Phase 3 Efficacy of sarilumab in combination with corticosteroid (CS short tapering regimen) in comparison to placebo (CS long tapering regimen) in patients with Polymyalgia Rheumatica	280	• Patients suffering from PMR, • Randomized, parallel assignment, double-blind, placebo controlled, 2 groups: sarilumab + CS, placebo +CS • Study duration per patient: approximately 62 weeks: up to 4 weeks screening, 52-week Tx period, 6-week follow-up period	• Primary: % of patients achieving sustained remission at week 52 • Secondary: components of sustained remission, cumulative corticosteroid dose, time to 1st PMR flare, change in glucocorticoid toxicity index, AEs, PK,	• SSD: Nov. 2018 • DE: 2022

SAR440340 (Anti-IL33 mAb)
COPD

Study	Description	Patients	Design	Endpoints	Status
POC in COPD ACT15104 NCT03546907	Phase 2 Efficacy, Safety and Tolerability (POC) of SAR440340 in patients with moderate-to-severe COPD	343	• Adults patients with a diagnosis of moderate-to-severe COPD for at least 1 year • Randomized, Double-blind, Placebo-controlled, on top of standards of care • Arm 1: SAR440340 • Arm 2 : placebo • Total duration for one patient: 46 to 76 weeks including 10 days to 4 weeks of screening, 24 to 52 weeks Tx period and 20 weeks post IMP Tx period	• Primary: AECOPD (Acute Exacerbations in COPD) • Secondary: average change in pre-bronchodilator FEV1 (forced expiratory volume 1), time to 1st COPD exacerbations, AEs, change in post-bronchodilator FEV1	• SSD: Jul. 2018 • DE: 2020

SAR441236 (Tri-specific neutralizing mAb)
HIV

Study	Description	Patients	Design	Endpoints	Status
HIV TDU15867 NCT03705169	Phase 1 Pharmacokinetics of SAR441236 in Participants with HIV	60	• Patients with HIV infection, • Randomized, Double-blind, Parallel-Group, Ascending dose study, • Arm A cohort 1A: SAR441236 (1mg/kg) + ART (antiretroviral Tx) from D0, • Arm A cohort 1B: placebo + ART from D0, • Arm A cohort 2A: SAR441236 (3mg/kg) + ART from D0, • Arm A cohort 2B: placebo + ART from D0 • Arm A cohort 3A: SAR441236 (10mg/kg) + ART from D0, • Arm A cohort 3B: placebo + ART from D0, • Arm A cohort 4A: SAR441236 (30mg/kg) + ART from D0, • Arm A cohort 4B: placebo + ART from D0, • Arm B cohort 5: SAR441236 (1mg/kg) and ART initiated at D28, • Arm B cohort 6: SAR441236 (3mg/kg) and ART initiated at D28, • Arm B cohort 7: SAR441236 (10 mg/kg) + ART from D28, • Arm B cohort 8: SAR441236 (30 mg/kg) + ART from D28, • Arm B cohort 9: SAR441236 (0,3 mg/kg) + ART from D28,	• Primary: Occurrence of a Grade 3 or higher AE (DAIDS AE grading table), at any time, AUC12w, change in plasma HIV-1 RNA (Arm B cohorts) • Secondary: change in plasma HIV-1 RNA (Arm B cohorts) at different times, maximum reduction of plasma HIV-1 RNA, SAR441236 Antibodies, change in CD4+T cell counts, SAR441236 PK.	• SSD: April 2019 • DE: 2021

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

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

Study	Description	Patients	Design	Endpoints	Status
CD38+HM TED10893 NCT01084252	Phase1/2 Dose escalation, Pharmacokinetics and efficacy study of isatuximab in patients with selected CD38+ HM	351 (enrollment completed)	• Phase 1: MTD • Phase 2: Stage 1: isatuximab activity at different doses/schedules and to select dose and regimen as single agent or in combination with dexamethasone Stage 2: activity at the selected dose/schedule from stage1, as single agent (ISA arm) and in combination with dexamethasone (ISAdex arm) • Randomized, Open-label, Parallel assignment	• Primary: DLT, ORR • Secondary: DOR, PFS, OS, Immune Response	• SSD: Jun. 2010 • DE: Primary completion:2018; Full completion: 2020

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

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

Study	Description	Patients	Design	Endpoints	Status
CD38+HM TED15085 NCT03733717	Phase1 Pharmacokinetics Safety and Preliminary Efficacy of isatuximab in Chinese Patients with Relapsed and/or Refractory MM	25 (actual)	• In Patients with known diagnosis of symptomatic multiple myeloma, • Open-label, Single Group assignment • Isatuximab every week in Cycle 1 (4 weeks) followed by every 2 weeks (Q2W) in subsequent cycles	• Primary: Pharmacokinetics • Secondary: Aes, ORR, DOR, TTP, PFS, OS, immunogenicity	• SSD: Oct. 2018 • DE: Primary completion: 2020; Full completion 2021

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

Study	Description	Patients	Design	Endpoints	Status
Lenalidomide Combination **RRMM** TCD11863 NCT01749969	Phase 1b Isatuximab, in Combination With lenalidomide and dexamethasone for the Tx of Relapsed or Refractory MM	57 (enrollment completed)	• Patients with diagnosis of MM and documentation of at least 2 prior therapies (induction therapy, autologous stem cell transplant, consolidation and maintenance therapy is considered one prior therapy) • Open-label, Parallel assignment • Isatuximab (escalating doses) + lenalidomide + dexamethasone • Total duration for one patient: up to 21 days screening, at least 4 weeks Tx, up to 60 days follow-up	• Primary: N of patients with AE • Secondary: ORR, PFS, PK, PD, Immunogenicity	• SSD: Feb. 2013 • DE: 2020

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

Study	Description	Patients	Design	Endpoints	Status
Pomalidomide Combination **RRMM** TCD14079 NCT02283775	Phase 1b Isatuximab, in combination with pomalidomide and dexamethasone for the Tx of Relapsed/Refractory MM	92 (enrollment completed: 45 patients in Part A; 47 patients in Part B)	• Patients previously diagnosed with MM based on standard criteria and currently require Tx because MM has relapsed following a response • Open-label, Single-Group assignment • Isatuximab + pomalidomide + dexamethasone • Part A, doses ranging for isatuximab, (5mg/kg, 10mg/kg, 20mg/kg); Part B isatuximab (10mg/kg) from a fixed infusion volume	• Primary: DLTs, N of patients with AE • Secondary: ORR, PK, Immunogenicity, DOR, CB	• SSD: May 2015 • DE: 2021

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

Study	Description	Patients	Design	Endpoints	Status
Bortezomib Combination **NDMM** TCD13983 NCT02513186	Phase 1 Isatuximab in combination with bortezomib - based regimens in adult patients with newly diagnosed MM non eligible for transplantation or with no intent for immediate transplantation	88 (17 pts in VCdI, 27 pts in VRdI cohort A, 44 pts in cohort B)	• 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 VCDI cohort: MTD and Recommended Dose (RC), based on DLTs, ORR and CR; • Primary VRDI cohort: CR • Secondary: overall safety profile, PK, isatuximab immunogenicity, ORR, PFS, AE and tumor response, infusion duration, MRD in patients achieving CR or VGPR	• SSD: Sep. 2015 • DE: 2023

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

Study	Description	Patients	Design	Endpoints	Status
RRMM TED14154 NCT02514668	Phase 1 Safety, PK and Efficacy of isatuximab in patients with Relapsed/Refractory MM	58 (enrollment completed)	• Patients with a diagnosis of MM with evidence of measurable disease and with evidence of disease progression • Open-label, Single Group assignment, isatuximab (escalating doses) • Total duration for one patient: up to 21 days screening, Tx period up to disease progression or AEs , 60-day follow-up at least	• Primary: Part A: DLTs, N of patients with AE; Part B: ORR • Secondary: PK, N of patients with AEs, DOR, CB, PFS, Immunogenicity	• SSD: Sep. 2015 • DE: 2020

Isatuximab (anti-CD38 mAb)
Relapsed/Refractory Multiple Myeloma (RRMM)

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

Study	Description	Patients	Design	Endpoints	Status
RRMM TCD15484 NCT04045795	Phase 1b Pharmacokinetics, Safety and Efficacy of isatuximab (SC and IV) in combination with Pomalidomide and Dexamethasone in patients with Relapsed/Refractory MM	46	• Patients with a diagnosis of MM based on standard criteria and requiring Tx because of a relapse following a response, • Open-label, Randomized, Sequential assignment, • 5 arms, each in combination with pomalidomide and dexamethasone: isatuximab SC (3 dose levels) and isatuximab IV (2 dose levels) • Total study duration: approximately 14 months: 21 days screening, Tx period until disease progression, unacceptable adverse reaction or other reason for discontinuation; FU: 30 days	• Primary: AEs, PK parameters, • Secondary: bioavailability, OOR, DOR, TTR, TTP, OS, CBR, PFS, patients expectations and satisfaction, Immunogenicity	• SSD: Aug. 2019 • DE: Primary: 2021; Full completion: 2022

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

Study	Description	Patients	Design	Endpoints	Status
ISLANDS (Japanese Patients) **RRMM** TED14095 NCT02812706	Phase 1 Phase 2 Isatuximab single-agent in Japanese patients with Relapsed and Refractory MM	36 (enrollment completed)	• Patients with a diagnosis of symptomatic MM, having received at least 3 prior lines of therapy OR whose disease is double refractory to an IMiD and a PI • Open-label, Single Group assignment, isatuximab monotherapy • Total duration for one patient: up to 21-day screening, Tx period up to disease progression or unacceptable AEs, post-Tx follow-up	• Primary: Phase 1: DLTs Phase 2: ORR • Secondary: N of patients with AE, CB, OS, PFS, DOR, TTR, PK, PD, Immunogenicity	• SSD: Sep. 2016 • DE: primary completion 2018; full completion 2020

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

Study	Description	Patients	Design	Endpoints	Status
Cemiplimab Combination **RRMM** TCD14906 NCT03194867	Phase 1 Phase 2 Safety, PK and Efficacy of isatuximab in combination with cemiplimab in patients with Relapsed/Refractory MM	109	• Patients with a diagnosis MM with evidence of measurable disease, having received prior Tx with an IMiD and with at least 3 prior lines of therapy • Randomized, Open-label, Parallel Assignment • Isatuximab + cemiplimab • 3 Arms: Isa +cemi regimen 1; isa + cemi regimen 2; isa alone • Total duration for one patient: up to 21-day screening, Tx period up to disease progression or unacceptable AEs, 3-month post-Tx follow-up. Cycle duration 28 days	• Primary: DLTs, N of patients with AE, ORR • Secondary: CB, DOR, TTR, PFS, OS, PK, Immunogenicity (isatuximab and cemiplimab)	• SSD: Feb. 2018 • DE: 2021

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

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

Study	Description	Patients	Design	Endpoints	Status
ICARIA-MM RRMM EFC14335 NCT02990338	Phase 3 Isatuximab, pomalidomide, and dexamethasone to pomalidomide and dexamethasone in Refractory or Relapsed and RRMM	307 (enrollment completed)	• Isatuximab in combination with pomalidomide and low-dose dexamethasone, compared to pomalidomide and low-dose dexamethasone in patients with RRMM • Randomized, Open-label, Parallel assignment	• Primary: PFS • Secondary: ORR, OS, TTP, PFS, DOR, safety, PK profile, immunogenicity	• SSD: Jan. 2017 • DE[1]: Final completion 2021

(1) Final Data Collection date for primary outcome measure

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

Study	Description	Patients	Design	Endpoints	Status
IKEMA RRMM EFC15246 NCT03275285	Phase 3 Isatuximab combined with carfilzomib and dexamethasone vs. carfilzomib with dexamethasone in patients With Relapse and/or Refractory MM previously treated with 1 to 3 prior lines	302 (enrollment completed)	• 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, TTP, PFS2, AE, PK, Immunogenicity	• SSD: Oct. 2017 • DE: Primary: 2020, Full completion: 2023

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

Study	Description	Patients	Design	Endpoints	Status
IMROZ NDMM Ti EFC12522 NCT03319667	Phase 3 Isatuximab in combination with bortezomib (Velcade®), lenalidomide (Revlimid®) and dexamethasone vs. bortezomib, lenalidomide and dexamethasone in patients with newly diagnosed MM not eligible for transplant	483 (randomized)	• Newly diagnosed MM not eligible for transplant due to age (≥ 65 years) or patients < 65 years with comorbidities impacting possibility of transplant • Randomized, Open-label, Parallel assignment • IVRd arm (Isatuximab/bortezomib/lenalidomide /dexamethasone) • VRd arm (Bortezomib/lenalidomide /dexamethasone) • Ird crossover arm (Isatuximab/lenalidomide/ dexamethasone) • Total duration for each patient: screening period up to 4 weeks, induction period of 24 weeks, continuous Tx period and crossover when applicable	• Primary: PFS • Secondary: ORR, % of patients with CR, and VGPR, % of patients with MRD (Minimal Residual Disease) negative, OS, TTP, DOR, PFS on next line of therapy (PFS2), AE, PK, Immunogenicity, QOL	• SSD: 2017 • DE: Primary: 2022, Full completion: 2025

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

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

Isatuximab (anti-CD38 mAb)
Pediatrics: RR ALL/AML

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

Study	Description	Patients	Design	Endpoints	Status
Pediatrics ALL/AML ACT15378 NCT03860844	Phase 2 Anti-tumor Activity, Safety and Pharmacokinetics of isatuximab in combination with Chemotherapy in Pediatric Patients from 28 days to less than 18 years of Age with Relapsed/Refractory B or T Acute Lymphoblastic Leukemia or Acute Myeloid Leukemia in First or Second Relapse	96	• Open-label, Single-group assignment • 2 cohorts: AML & ALL, in combination with chemotherapy	• Primary: CR in AML, B-ALL or T-ALL, •Secondary: AE²s, incidence and severity of infusion reactions, isatuximab PK, minimal residual disease, ORR, OS, Event free survival, DR, Relationship between clinical effects and CD38 receptor density and occupancy	• SSD: Apr. 2019 • DE: 2022

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

Study	Description	Patients	Design	Endpoints	Status
Advanced Malignancies ACT15319 NCT03367819	Phase 1/2 Safety and tolerability of Isatuximab in combination with cemiplimab in patients with metastatic castration-resistant prostate cancer (mCRPC) or patients with non-small cell lung cancer (NSCLC)	134	• In Patients with metastatic, castration-resistant prostate cancer (mCRPC) who are naïve to anti-programmed cell death-1 (PD-1)/programmed cell death-ligand 1 (PDL-1)-containing therapy, or non-small cell lung cancer (NSCLC) who progressed on anti-PD-1/PDL-1-containing therapy, • Randomized, Open-Label, Parallel Assignment • Isatuximab alone or in combination with cemiplimab • Total duration per patient up to 28 months including 28 days screening period, , up to 24 months ttmt period and 3 months safety FU	• Primary: Safety, tolerability, RR • Secondary: Immunogenicity (isa and cemi), PK, tumor burden change, DR, PFS, Disease Control Rate	• SSD: Jan. 2018 • DE: Full completion safety : 2021

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

Study	Description	Patients	Design	Endpoints	Status
Advanced Malignancies ACT15320 NCT03769181	Phase 1/2 Safety, Preliminary Efficacy and Pharmacokinetics of Isatuximab in combination with cemiplimab in patients with Lymphoma	130	• In Patients with Lymphoma: *Cohort A1*: classic Hodgkin'Lymphoma (cHL) anti-PD-1/PD-L1 inhibitor naïve, *Cohort A2*: cHL) anti-PD-1/PD-L1 inhibitor progressor *Cohort B*: diffuse large B-cell Lymphoma (DLBCL) *Cohort C*: peripheral T-cell Lymphoma (PTCL) • Non-Randomized, Open-Label, Parallel Assignment • Isatuximab in combination with cemiplimab	• Primary: *Phase 1*: DLTs, recommended Phase 2 dose (RP2D), *Phase 2*: Cohort A1: Complete Remission Rate (CRR); Cohort A2 RR • Secondary: AEs, SAEs, PK, tumor burden, disease control rate, DR, PFS	• SSD: Jan. 2019 • DE: Primary: 2023, Full completion: 2023

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

Study	Description	Patients	Design	Endpoints	Status
Advanced Malignancies ACT15377 NCT03637764	Phase 1/2 Safety, Preliminary Efficacy and Pharmacokinetics of Isatuximab monotherapy or in combination with Atezolizumab in patients with Advanced Malignancies	350	• In Patients with a known diagnosis of either unresectable HCC, platinum-refractory /recurrent /metastatic SCCHN, platinum-resistant/refractory EOC with evidence of measurable disease or recurrent GBM, • Non-Randomized, Open-Label, Parallel Assignment, • Isatuximab alone or in combination with atezolizumab,	• Primary: DLTs, AEs, MTD, Recommended Phase 2 dose, RR, PFS, • Secondary: immunogenicity (Isatuximab and atezolizumab), tumor burden change, disease control rate, DR, PFS, RR, PK,	• SSD: Aug. 2018 • DE: Primary:2021, Full completion: 2023

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

Study	Description	Patients	Design	Endpoints	Status
mCRC Umbrella Trial led by Roche For sanofi: ACT16241 NCT03555149	Phase 1b/2 Efficacy and Safety, of isatuximab in combination with atezolizumab in patients with Metastatic Colorectal Cancer	326	• Umbrella study, Randomized, Open-Label, Parallel Assignment, • Isatuximab in combination with atezolizumab, • Patients will receive Tx until unacceptable toxicity or loss of clinical benefit as confirmed by disease progression or lack of continued benefit as determined by the investigator	• Primary: ORR, AEs • Secondary: PFS, OS, DOR, % of patients alive at Month 6, DCR, immunogenicity,	• SSD: Sep. 2018 • DE: 2022

Isatuximab (anti-CD38 mAb) Combination Lenalidomide and Dexamethasone – High-risk Smoldering Multiple Myeloma

Study	Description	Patients	Design	Endpoints	Status
EFC15992 NCT04270409	Phase 3 A Phase 3 Randomized, Open Label, Multicenter Study of Isatuximab (SAR650984) in Combination With Lenalidomide and Dexamethasone Versus Lenalidomide and Dexamethasone in Patients With High-risk Smoldering Multiple Myeloma	300	• Isatuximab in combination Lenalidomide and Dexamethasone	Primary: • safety run-in: To confirm the recommended dose of isatuximab when combined with lenalidomide and dexamethasone in participants with high-risk smoldering multiple myeloma (SMM) • Randomized Phase 3: To demonstrate the clinical benefit of isatuximab in combination with lenalidomide and dexamethasone in the prolongation of progression-free survival when compared to lenalidomide and dexamethasone in subjects with high-risk SMM	• SSD: May 2020 • DE: Primary: 2027, Full: 2033

Isatuximab (anti-CD38 mAb) – Kidney Transplant

Study	Description	Patients	Design	Endpoints	Status
TED16414 NCT04294459	Phase 1b/2 A Phase 1b/2 Study to Evaluate the Safety, Pharmacokinetics, and Preliminary Efficacy of Isatuximab (SAR650984) in Patients Awaiting Kidney Transplantation	42	• screening period of up to 28 days, • treatment period of up to 12 weeks • study duration of approximately 42 weeks. • study duration including extended FUP per participant will be approximately 78 weeks	Primary : • Phase 1: safety and tolerability of isatuximab in kidney transplant candidates. • Phase 2: efficacy of isatuximab in desensitization of patients awaiting kidney transplantation. Secondary : • Phase 2: safety profile of isatuximab in kidney transplant candidates. • pharmacokinetic (PK) profile of isatuximab in kidney transplant candidates. • immunogenicity of isatuximab. • overall efficacy of isatuximab in desensitization of patients awaiting kidney transplantation.	• SSD: May 2020 • DE: 2024

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

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

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

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

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

Cemiplimab (PD-1 inhibitor)
Pediatrics

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

Study	Description	Patients	Design	Endpoints	Status
RR Solid tumors CNS tumors ND R Glioma R2810-ONC-1690 NCT03690869	Phase 1 Phase 2 a) Safety and Pharmacokinetics of cemiplimab single agent in Pediatric Patients with Relapsed Refractory Solid or CNS Tumors b) Safety and Efficacy of cemiplimab in combination with Radiotherapy in Pediatric Patients with Newly Diagnosed Diffuse Intrinsic Pontine Glioma, Newly Diagnosed High-Grade Glioma or Recurrent High-Grade Glioma	150	• Randomized, Parallel Group assignment, Open-label • Phase1: cemiplimab monotherapy in both cohorts: Solid Tumor and CNS cohorts • Phase 2: Newly Diagnosed DIPG, Newly Diagnosed HGG, recurrent HGG: cemiplimab in combination with radiation therapy	• Primary: DLTs (Phase 1 & 2), Anticipated recommended dose from Phase 1 to Phase 2, cemiplimab PK (monotherapy and in combination with radiation therapy), anticipated cemiplimab RP2D when co-administered with radiation therapy in DIPG and HGG, anti-tumor activity: OS12, PFS12, • Secondary: anti-tumor activity (children objective response), immunogenicity, tolerability profile (DLTs & AEs)	• SSD: Early 2019 • DE: 2025

Cemiplimab (PD-1 inhibitor)
Melanoma - Biomarkers

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

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	47 (actual)	• For Histologically confirmed diagnosis of stage III (unresectable) or stage IV cutaneous melanoma (non-acral lentiginous) with at least 1 lesion that is measurable by RECIST 1.1 criteria and accessible for biopsies	• 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: 2020

Cemiplimab (PD-1 inhibitor)
Head and Neck - Biomarkers

Study	Description	Patients	Design	Endpoints	Status
Biomarkers Head & Neck R2810-ONC-1655 NCT03198130	Phase 1 Exploratory Tumor Biopsy-driven study to understand the relationship between biomarkers and clinical response in Immunomodulatory Treatment-Naïve patients with Recurrent and/or Metastatic Squamous Cell Carcinoma of Head and Neck receiving cemiplimab	33 (actual)	• For Histologically confirmed diagnosis recurrent and/or metastatic SCCHN (squamous cell carcinoma of the head and neck) with no curative options with at least 1 lesion that is measurable by Response Evaluation Criteria in Solid Tumors (RECIST) • Open-label, Single Group Assignment	• Primary: Correlation between changes in the tumor microenvironment and the change in tumor volume following cemiplimab Tx • Secondary: Correlation between baseline tumor characteristics and the change in tumor volume following Tx, ORR, PFS, TAES, cemiplimab serum concentration, anti-cemiplimab antibodies level	• SSD: Jul. 2017 • DE (1st Part) (1): 2019; full completion 2020

(1) Final Data Collection date for primary outcome measure

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

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

Study	Description	Patients	Design	Endpoints	Status
Advanced CSCC R2810-ONC-1540 NCT02760498	Phase 2 Cemiplimab monotherapy for patients with metastatic (nodal or distant) CSCC (Groups 1 and 3) or with unresectable locally advanced CSCC (Group 2)	266	• Non-Randomized, Open-label, Parallel assignment • Group 1: Patients with metastatic CSCC: to distant sites or lymph nodes cemiplimab administered intravenously every 2 weeks • Group 2: Patients with unresectable locally advanced CSCC. cemiplimab administered intravenously every 2 weeks • Group 3: Patients with metastatic CSCC: to distant sites or lymph nodes, cemiplimab administered intravenously every 3 weeks • Group 4: Patients with advanced CSCC , metastatic (nodal or distant) or unresectable locally advanced , cemiplimab administered every 4 weeks • Group 5: Patients in advanced CSCC receiving a single SC dose of cemiplimab, followed by cemiplimab IV Q3W (pilot Group)	• Primary: ORR (96 weeks), Groups 1,3 and 4: RECIST version 1.1 will be used to determine ORR, Group 2 and 4: Clinical response criteria will be used to determine ORR • Secondary: Investigator Assessments of ORR, DOR, PFS, OS, CRR, cemiplimab PK and antibodies levels, patients reported outcomes (EORTC QLQ-C30), TEAEs	• SSD: May 2016 • DE: Primary:2020; Full completion 2021

Cemiplimab (PD-1 inhibitor)
Neoadjuvant CSCC

Study	Description	Patients	Design	Endpoints	Status
Neoadjuvant CSCC R2810-ONC-1787 NCT03889912	Phase 1 Study of Pre-Operative cemiplimab administered Intralesionally, for Patients with Recurrent Cutaneous Squamous Cell Carcinoma (CSCC)	36	• Patients with history of recurrent resectable CSCC • Open-label, Single-Group assignment • Three dose cohorts planned followed by a 3+3 dose-escalation design with cohort expansion	• Primary: DLTs, TEAs, injection site reactions, • Secondary: ORR, pathologic complete response rate, major pathologic response rate, cemiplimab serum concentration, cemiplimab antibodies, selection of the recommended cemiplimab dose for further study based on clinical and PK observations.	• SSD: Apr. 2019 • DE: 2020

Cemiplimab (PD-1 inhibitor)
Neoadjuvant CSCC post surgery

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

Study	Description	Patients	Design	Endpoints	Status
Neoadjuvant CSCC R2810-ONC-1788 NCT03969004	Phase 3 Adjuvant Cemiplimab vs Placebo after Surgery and Radiation Therapy in Patients with High risk CSCC	412	• Patients with resection of pathologically confirmed CSCC, and qualified as High Risk CSCC, • Randomized, placebo-controlled, double-blind, parallel assignment, • 2 arms: cemiplimab and placebo,	• Primary: DFS (time from randomization to the first documented disease recurrence) • Secondary: OS, FFLRR (from randomization to the 1st locoregional recurrence LRR), FFDR (from randomization to the 1st distant recurrence), cumulative occurrence of second primary CSCC, TEAEs, incidence of deaths, lab. abnormalities,	• SSD: June 2019 • DE: Primary: 2023, Full completion: 2026

Cemiplimab (PD-1 inhibitor)
Neoadjuvant CSCC

Study	Description	Patients	Design	Endpoints	Status
Neoadjuvant CSCC R2810-ONC-1901 NCT04154943	Phase 2 Neodjuvant Cemiplimab for Stage II to IV CSCC	76	• Patients with Stage II to IV CSCC, • Open-label, single group assignment (cemiplimab)	• Primary: pCR (pathologic Complete Response), • Secondary: mPR (Major Pathologic response, pCR, mPR, ORR, EFS, DFS, OS, incidence of deaths, laboratory abnormalities, change in surgical plan and in post chirurgical plan.	• SSD: Q1 2020 • DE: Primary: 2021, Full completion: 2025

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

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

Study	Description	Patients	Design	Endpoints	Status
BCC R2810-ONC-1620 NCT03132636	Phase 2 Cemiplimab in patients with Advanced BCC who experienced progression of disease on Hedgehog Pathway Inhibitor Therapy, or were intolerant of Prior Hedgehog Pathway Inhibitor Therapy	137	• Patients with confirmed diagnosis of invasive BCC • Non-Randomized, Open-label, Parallel assignment • Group 1: Patients with metastatic BCC • Group 2: Patients with unresectable locally advanced BCC	• Primary: ORR for mBCC measured by RECIST version 1.1 ORR for unresectable locally advanced BCC measured by Composite Response Criteria • Secondary: DOR, CR, PFS, OS, TEAEs, PK, immunogenicity	• SSD: July 2017 • DE: Primary: 2021, Full completion 2022

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

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

Study	Description	Patients	Design	Endpoints	Status
mNSCLC R2810-ONC-1624 NCT03088540	Phase 3 First-line Tx in patients with advanced or metastatic NSCLC whose tumors express PD-L1, vs. Platinum Based Chemotherapy	700	• For histologically or cytologically documented squamous or non squamous NSCLC with stage IIIB or stage IIIC who are not candidates for Tx with definitive chemoradiation or patients with stage IV disease who received no prior systemic Tx for recurrent or metastatic NSCLC • Randomized, Open-label, Cross-over assignment • Active Comparator: Standard-of-care chemotherapy: paclitaxel + cisplatin OR paclitaxel + carboplatin OR gemcitabine + cisplatin or gemcitabine + carboplatin OR Pemetrexed + cisplatin followed by optional pemetrexed maintenance OR pemetrexed + carboplatin followed by optional pemetrexed maintenance	• Primary: OS, PFS as assessed by a blinded Independent review committee using RECIST 1.1 • Secondary: Objective response rates, BOR, DOR	• SSD: May 2017 • DE: 2023

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

Study	Description	Patients	Design	Endpoints	Status
mNSCLC R2810-ONC-16113 NCT03409614	Phase 3 Combination of cemiplimab and Platinum-based Doublet Chemotherapy in patients with Lung Cancer	Part 1: 360 Part 2: 450	• For histologically or cytologically documented squamous or non squamous NSCLC with stage IIIB or IIIC disease who are not candidates for Tx with definitive concurrent chemoradiation or stage IV disease who received no prior systemic Tx for recurrent or metastatic NSCLC • **Part 1**: Randomized, Open-label, Parallel assignment • Arm 1: Standard of care Platinum-based doublet chemotherapy • Arm 2: cemiplimab + Platinum-based doublet chemotherapy • Arm 3: cemiplimab + abbreviated chemotherapy + ipilimumab • **Part 2**: Randomized, Double-Blind, • Arm 1: Standard of care Platinum-based doublet chemotherapy • Arm 2: cemiplimab + standard of care Platinum-based doublet chemotherapy	• Primary: Part 1: ORR; Part 2: OS and PFS as assessed by a blinded independent review committee using RECIST1.1, • Secondary: TEAEs, DLTs, SAEs, incidence of deaths, laboratory abnormalities, QoL	• SSD: Mar. 2018 • DE: 2023

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

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

Study	Description	Patients	Design	Endpoints	Status
mNSCLC R2810-ONC-16111 NCT03515629	Phase 3 Combination of cemiplimab, Platinum-based Doublet Chemotherapy, and ipilimumab vs pembrolizumab in Patients with Lung Cancer	5*	• For histologically or cytologically documented squamous or non squamous NSCLC with stage IIIB or stage IV disease who received no prior systemic Tx for recurrent or metastatic NSCLC • Randomized, Open-label, Parallel assignment • Arm 1: pembrolizumab • Arm 2: cemiplimab + ipilimumab • Arm 3: cemiplimab + chemotherapy + ipilimumab	• Primary: PFS as assessed by a blinded Independent review committee using RECIST 1.1 • Secondary: OS, ORR, TEAEs, DLTs, SAEs, death, lab. abnormalities, OS, QoL	• SSD: June 2018 • DE: 2020

*: study ongoing with the patients included but recruitment stopped

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

Study	Description	Patients	Design	Endpoints	Status
mNSCLC R2810-ONC-1763 NCT03430063	Phase 2 Cemiplimab and Ipilimumab in Patients with Lung Cancer	28*	• For histologically or cytologically documented squamous or non squamous NSCLC with stage IIIB or IIIC and not candidates for definitive chemoradiation or stage IV. Patients must have PD after receiving one prior line of chemotherapy Tx for advanced NSCLC, • Randomized, Open-label, Parallel assignment • Arm 1: cemiplimab standard dose • Arm 2: cemiplimab + ipilimumab standard doses • Arm 3: cemiplimab High dose	• Primary: ORR • Secondary: OS, PFS, TEAEs, SAEs, death, lab. abnormalities	• SSD: May 2018 • DE: 2020

*: study ongoing with the patients included but recruitment stopped

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

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

Study	Description	Patients	Design	Endpoints	Status
CC R2810-ONC-1676 NCT03257267	Phase 3 Cemiplimab vs. therapy of Investigator Choice chemotherapy in Recurrent or Metastatic CC	534	• Patients with recurrent, persistent and/or metastatic CC with squamous cell histology for which there is no curative intent option, • Randomized, Open-label, Parallel assignment, Tx cycle 6 weeks, Planned Tx for up to 96 weeks • 2 arms: cemiplimab and Investigator Choice (IC) chemotherapy	• Primary: OS • Secondary: PFS, ORR, DOR, QOL	• SSD: Oct. 2017 • DE: Primary: 2020; Next 2021; Full completion 2023

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

Study	Description	Patients	Design	Endpoints	Status
AST **Monotherapy and combination with cemiplimab** TCD14678 NCT03192345	Phase 1/1b Safety, PK, PD and Anti-tumor activity of SAR439459 Monotherapy and in combination with cemiplimab in adult patients with Advanced Solid Tumors	225	• Patients with histologically confirmed, advanced unresectable or metastatic solid tumor • Open-label, Parallel assignment • Part 1A: SAR439459 monotherapy escalating doses • Part 2A: SAR439459 monotherapy with the previously recommended dose • Part 1B: SAR439459 escalating dose + cemiplimab standard dose • Part 2B: SAR439459 at previously recommended dose + cemiplimab standard dose • Escalation periods non randomized followed by expansion periods randomized	• Primary: incidence of DLTs (Part 1), ORR (Part 2) • Secondary: Safety profile, Immunogenicity, PK, PFS (Part 2), TTP (Part 2)	• SSD: Jun. 2017 • DE: Primary (melanoma): 2021; Full completion: 2022

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

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

Study	Description	Patients	Design	Endpoints	Status
First-in-Human TED13751 NCT02187848	Phase 1 Phase 2 Safety, PK and antitumor activity of SAR408701 in patients with AST	337	• Patients with locally advanced or metastatic solid malignant tumor • Non-Randomized, Open-label, Parallel assignment • Arm 1 : SAR408701 monotherapy escalating cohorts • Arm 2: SAR408701 expansion cohort in CRC with MTD previously defined • Arm 3: SAR408701 expansion cohort in non-squamous NSCLC high expresser patients (CEACAM5 >50% of tumor cells ≥ 2+ intensity) at MTD • Arm 4: SAR408701 expansion cohort gastric adenocarcinoma at MTD • Arm 5: SAR408701 loading dose at first cycle followed by MTD • Arm 6: SAR408701 expansion cohort in non-squamous NSCLC patients (Lung bis) with CEACAM5 >1% of tumors cells ≥ 2+ intensity, at MTD • Arm 7: SAR408701 expansion cohort SCLC at MTD • Arm 8: SAR408701 expansion cohort CRC-L at MTD • Arm 9: SAR408701 dose escalation every 3 weeks	• Primary: MTD, Anti-tumor response RECIST • Secondary: Overall Safety, Immunogenicity, PK, duration of response, time to progression	• SSD: Sep. 2014 • DE: 2021

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

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

Study	Description	Patients	Design	Endpoints	Status
Japanese patients **Monotherapy** TCD15054 NCT03324113	Phase 1 Safety and PK of SAR408701 Monotherapy in Japanese patients with Advanced Malignant Solid Tumors	24 (expected)	• Patients with advanced or metastatic malignant solid tumor • Open-label, Sequential assignment • 14-day cycle • Amendment to test loading dose ongoing	• Primary: DLTs, • Secondary: Safety, Immunogenicity, PK, Plasma CEACAM5 levels, Anti-tumor response RECIST	• SSD: Oct. 2017 • DE: 2021

SAR408701 (maytansin loaded anti-CEACAM5 mAb) NSCLC

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

Study	Description	Patients	Design	Endpoints	Status
CARMEN-LC03 EFC15858 NCT04154956	Phase 3 SAR408701 vs Docetaxel in patients with Previously Treated Metastatic Non-Squamous NSCLC with CEACAM5 positive tumors	554	• Patients with histologically or cytologically proven diagnosis of non-squamous NSCLC, with metastatic disease progression after platinum-based chemotherapy and immune checkpoint inhibitor, and with carcinoembryonic antigen-related cell adhesion molecule (CEACAM) 5 expression, • Randomized, Open-label, Parallel assignment • 2 Arms: SAR408701 & Docetaxel	• Primary: PFS, OS, • Secondary: ORR, Quality of life (disease related symptoms, physical function, role function), safety, DOR.	• SSD: Nov. 2019 • DE: 2024

SAR439859 (SERD)
Breast cancer (1/3)

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

Study	Description	Patients	Design	Endpoints	Status
TED14856 NCT03284957	Phase 1 Phase 2 SAR439859 single agent and in combination with palbociclib in Postmenauposal Women with Estrogen Receptor Positive Advanced Breast Cancer	259	• Non-Randomized, Open-label, Parallel Assignment • Part A: SAR439859 monotherapy dose escalation, • Part C: dose escalation for the combination SAR439859 and palbociclib, • Part B: SAR439859 dose expansion from the dose determined in part A, • Part D: combination SAR439859 and palbociclib at the doses recommended from part C • SAR439859 administered in 28-day cycle; palbociclib in 21-day cycle • Part E: midazolam drug-drug interaction sub-study to assess the effect of SERD on CYPA3	• Primary: Parts A & C:DLTs, Parts B & D: ORR; safety • Secondary: Safety, ORR, TTR, DCR, DR, PK for both drugs, CYP450 3A induction/inhibition, ER occupancy/PET imaging	• SSD: Sept. 2017 • DE: 2021
TED15954 NCT03816839	Phase 1 Safety, Efficacy, Pharmacokinetics and Pharmacodynamics Evaluation of SAR439859 single agent in Japanese Postmenopausal Women with ER positive and HER2 negative Advanced Breast Cancer	12	• Open-label, Single-Group Assignment • SAR439859, administered orally once daily as monotherapy in fasted or fed conditions	• Primary: :DLTs, • Secondary: AEs, Pharmacokinetics of SAR439859, ORR, CBR,DR, non-progression rate,	• SSD: Apr. 2019 • DE: 2020

SAR439859 (SERD)
Breast cancer (2/3)

Study	Description	Patients	Design	Endpoints	Status
ACT16105 NCT04059484	Phase 2 SAR439859 versus endocrine Monotherapy With Estrogen Receptor-positive, HER2-Negative Locally Advanced or metastatic Breast Cancer With prior Exposure to Hormonal Therapies	282	• In patients with histological or cytological diagnosis of adenocarcinoma of the breast, • Randomized, Open-label, Parallel Assignment • 2 Arms: SAR439859 and endocrine monotherapy as per physician's choice	• Primary: PFS, • Secondary: ORR, DCR, CBR, DOR, PFS, OS, PK, patient reported outcomes, overall safety profile	• SSD: 2019 • DE: Primary: 2021, full Completion: 2022

SAR439859 (SERD)
Breast cancer (3/3)

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

Study	Description	Patients	Design	Endpoints	Status
ACT16106 NCT04191382	Phase 2 Window study of SAR439859 versus Letrozole in Newly Diagnosed Pre-operative Postmenopausal patients With ER positive and HER2 Negative Primary Breast Cancer	126	• In patients with histological or cytological diagnosis of invasive breast adenocarcinoma, • Randomized, Parallel Assignment, single masking • 3 Arms: SAR439859 (two dose levels) and letrozole as active comparator.	• Primary: change in Ki67 (% of positive tumor cells tested by immunochemistry) • Secondary: Ki67≥ 50%, ER, safety, laboratory abnormalities,	• SSD: Jan. 2020 • DE: 2021

SAR440234 (T-cell engaging bispecific mAb)
Leukemia and Myelodysplastic Syndrome

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

Study	Description	Patients	Design	Endpoints	Status
TED15138 NCT03594955	Phase 1 Phase 2 SAR440234 single agent in patients with Relapsed or Refractory Acute Myeloid Leukemia (RR AML), B-cell Acute Lymphoblastic Leukemia (B-ALL) or High Risk Myelodysplasia (HR-MDS)	77	• Patients with confirmed diagnosis of AML (except acute promyelocytic leukemia) or MDS with a risk category intermediate or higher, and not eligible for any Tx known to provide clinical benefit, • Open-label, Single Group Assignment • 2 dose escalation schemes, • Cycle defined as 6 weeks of study Tx • Tx may be continued as long as it is clinically beneficial	• Primary: DLTs, allergic reactions/hypersensitivity, ORR, DOR, event-free survival • Secondary: AEs, PK, Preliminary Anti-Leukemia Activity, immunogenicity	• SSD: Nov. 2018 • DE: 2022

FDA clinical hold lifted in August 2019 - Sites re-initiation activities ongoing .

SAR442720 (SHP2 inhibitor)
Relapsed/Refractory Solid Tumors

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

Study	Description	Patients	Design	Endpoints	Status
RMC-4630-01 NCT03634982	Phase 1 Safety, Tolerability, PK and PD profiles of SAR442720 single agent in patients with Relapsed/Refractory Solid Tumors	240	• Patients with advanced solid tumors that have failed, are intolerant or are considered ineligible for standard of care anticancer Tx • Open-label, Single Group Assignment • 1 Arm: SAR442720, oral administration	• Primary: AEs, DLTs, • Secondary: PK, pERK (PD markers), ORR, DOR,	• SSD: Oct. 2018 • DE: 2021
RMC-4630-02 NCT03989115	Phase 1 Phase 2 Safety, Tolerability, PK and PD profiles of SAR442720 and Cobimetinib in Adult participants with Relapsed/Refractory Solid Tumors With Specific Genomics Aberrations	144	• Patients with advanced solid tumors that have failed, are intolerant or are considered ineligible for standard of care anticancer Tx • Open-label, Single Group Assignment • 1 Arm: SAR442720 + Cobimetinib, oral administration	• Primary: AEs, DLTs, • Secondary: PK, ORR, DOR,	• SSD: July 2019 • DE: 2022

SAR441000 (Cytokine mRNA)
Advanced Solid Tumors

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

Study	Description	Patients	Design	Endpoints	Status
TED15297 NCT03871348	Phase 1 Safety, Pharmacokinetics, Pharmacodynamics and Anti-Tumor activity of SAR441000 as Monotherapy and in Combination with cemiplimab in patients with Advanced Solid Tumors	264	• Patients with advanced solid malignant tumors including lymphomas, for which no standard alternative therapy is available, • Non-randomized, Open-label, Parallel Assignment, • Dose escalation Phase, 2 arms: SAR441000 (intra-tumoral injection as monotherapy) and SAR441000 (intra-tumoral injection) + cemiplimab over a 21-day cycle, • Expansion cohorts in melanoma with SAR441000 monotherapy and with the combination (SAR441000 + cemiplimab), • Expansion cohorts in CSCC, HNSCC with the combination.	• Primary: DLTs (SAR441000 alone and in combination), MTD (SAR441000 alone and in combination), TEAEs, ORR for expansion, • Secondary: PK (SAR441000 alone and in combination), immunogenicity (SAR441000 and cemiplimab), DCR and DoR (SAR441000 alone and in combination), PFS, TEAEs, Recommended dose for SAR441000 alone and in combination for the expansion cohorts, ORR for dose escalation.	• SSD: Jan. 2019 • DE: 2021

SAR442085 (Anti CD38 mAb Fc engineered)
Multiple Myeloma

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

Study	Description	Patients	Design	Endpoints	Status
TED16132 NCT04000282	Phase 1 Safety, Pharmacokinetics, Pharmacodynamics and Anti-Tumor activity of SAR442085 in Patients with Relapsed or Refractory Multiple Myeloma	78	• Patients previously diagnosed with multiple myeloma based on standard criteria, • Non-randomized, Open-label, Sequential Assignment, • Part A: dose escalation • Part B: dose expansion	• Primary: MTD, recommended dose for Part B and further testing, ORR, • Secondary: TEAEs, PK, Anti-drug antibody, PFS, DR,	• SSD: Sept. 2019 • DE: 2022

SAR444245 - THOR 707
Solid Tumors

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

Study	Description	Patients	Design	Endpoints	Status
TCD16843 NCT04009681	Phase 1 An Open-Label, Multicenter Phase 1/2 Dose Escalation and Expansion Study of THOR-707 as a Single Agent and in Combination With a Checkpoint Inhibitor in Adult Subjects With Advanced or Metastatic Solid Tumors	300	• open-label • multiple ascending dose escalation and dose expansion study • Part 1: monotherapy • Part 2: combination with a checkpoint inhibitor	Primary: • Rate of Dose-Limiting Toxicities (DLTs) • Maximum Tolerated Dose (MTD) • Recommended Phase 2 Dose	• SSD: June 2019 • DE÷2022

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

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

Study	Description	Patients	Design	Endpoints	Status
COMET **Late Onset** EFC14028 NCT02782741	Phase 3 To compare efficacy and safety of Enzyme Replacement therapies avalglucosidase alfa (neoGAA) and alglucosidase alfa (Myozyme®/Lumizyme®) in Tx-naïve patients with Late-onset PD	102	• Repeated Biweekly Infusions of avalglucosidase alfa (GZ402666) and alglucosidase alfa in Tx-naïve patients with late-onset PD age 3 years and older • Randomized, Double-Blind, Parallel Assignment • Total study duration for one patient: 3 years [14-day screening, 49-week blinded Tx period, 96-week open-label Tx and 4-week post-Tx observation period	• Primary: Change in percent predicted forced vital capacity (%FVC) in the upright position, from baseline to 12 months • Secondary: Change from baseline to 12 months in six-minute walk test distance walked, maximal inspiratory / expiratory pressure (% predicted), hand-held dynamometry measurement of lower extremity muscle strength in Quick Motor Function Test scores, and 12- Item Short-form health survey scores	• SSD: Oct. 2016 • DE Primary: 2020 • DE Full Completion: 2024

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

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

Study	Description	Patients	Design	Endpoints	Status
Mini-COMET Infantile Onset ACT14132 **NCT03019406**	Phase 2 To assess safety and efficacy of avalglucosidase alfa (neoGAA) in Pediatric patients with infantile-onset PD previously treated With alglucosidase alfa (Myozyme®/Lumizyme®)	22	• Repeated bi-weekly infusions of avalglucosidase alfa In Patients with Infantile-onset PD previously treated with alglucosidase alfa (Myozyme®/Lumizyme®) who demonstrate clinical decline or sub-optimal clinical response • Randomized, Open-label, Ascending dose, Parallel assignment • Total study duration for one patient: 3 years [14-day screening, 25-week Tx period, a 120-week extension period and 4-week post-Tx observation period	• Primary: N of participants with AE, N of participants with immunogenicity response Secondary: PK parameters, Change at 6 months from baseline in Gross Motor Function (GMF) Measure-88 Test, revised GMF Classification System score, Pompe specific Pediatric Evaluation of Disability Inventory, Functional Skills Scale, Mobility Domain Test score and Quick Motor Function Test scores, Left Ventricular Mass Index, Eyelid position measurements, Creatine kinase value	• SSD: Oct. 2017 • DE Primary: 2019 • DE Full completion: 2022

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

Study	Description	Patients	Design	Endpoints	Status
NEO-EXT LTS13769 NCT02032524	Phase 2 Phase 3 Long-term safety and PK of repeated biweekly infusions of avalglucosidase alfa (neoGAA) in patients with PD	21	• Repeated biweekly infusions of avalglucosidase alfa In patients with PD who previously completed a avalglucosidase alfa study [adult, senior] • Non-randomized, Open-label, single group assignment • Total study duration for one patient: 6 years [until the patient withdraws, the Investigator withdraws the patient, or the Sponsor terminates the study]	• Primary: AEs and TEAEs, including IARs & deaths, Hematology, biochemistry and urinalysis, vital signs • Secondary: ECG, PK parameters, anti-avalglucosidase alfa antibodies, and neutralizing antibody formation in anti-avalglucosidase alfa positive patients, anti-alglucosidase alfa IgG antibodies, Skeletal muscle glycogen content, Qualitative and quantitative muscle degenerative assessments MRI, Urinary Hex4, plasma analyses of circulating mRNA and micro RNA, Serum analyses of skeletal muscle RNA expression	• SSD: Feb. 2014 • DE: 2021 (for post trial access)

Olipudase Alfa (rhASM ERT)
Acid Sphingomyelinase Deficiency (ASMD) (1/2)

Study	Description	Patients	Design	Endpoints	Status
ASCEND **Niemann-Pick disease type B**[1] DFI12712 NCT02004691	Phase 2 Phase 3 Efficacy, Safety, PD, and PK study of olipudase alfa in patients with ASMD	36	• Randomized, Double-blinded, Placebo-controlled, Parallel assignment • Study duration is composed of blinded period and an open label extension allowing patients that were on placebo to cross over to active treatment	• Primary: % change in spleen volume, % change in diffusing capacity of the lung for carbon monoxide (Dlco) • Secondary: Change in splenomegaly-related symptom score (except US, where it is part of the primary "combination spleen endpoint"), % change in liver volume, % change in platelet count, change in fatigue severity as measured by item 3 of the Brief Fatigue Inventory scale, **c**hange in pain severity as measured by item 3 of the Brief Pain Inventory scale, **c**hange in dyspnea severity as measured by the functional assessment of chronic illness therapy dyspnea tool	• SSD: June 2016 • DE: 2019[2] • DE: 2023[3]

(1) Non-neurological manifestations of ASMD
(2) Primary analysis period
(3) Completion of trial, end of long-term extension

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

Study	Description	Patients	Design	Endpoints	Status
Long-Term LTS13632 NCT02004704	Phase 2 Long-term study of olipudase alfa in patients with ASDM	20	• For patients who have completed a previous study with olipudase alfa (DFI13803 for pediatric patients, and DFI13412 for adult patients) • Open-label, Single group assignment • Total study duration for one patient: up to 9 years	• Primary: Safety parameters, complete physical examinations including neurologic examinations, vital signs, echocardiograms and electrocardiograms, clinical laboratory tests, safety biomarkers, immune response assessment, liver biopsy (patients previously enrolled in DFI13412) and liver ultrasound/doppler (patients previously enrolled in DFI13803). • Secondary: Spleen and liver volumes, pulmonary imaging and function tests, hematology and lipid profiles, health outcome questionnaires. For pediatrics patients: Hand X-ray for bone age and bone maturation, linear patient growth by height Z-score.	• SSD: Dec. 2013 • DE: 2023

Venglustat (GCS inhibitor)
Gaucher disease (GD) Type 3 (1/3)

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

Study	Description	Patients	Design	Endpoints	Status
LEAP GD Type 3 PDY13949 NCT02843035	Phase 2 Tolerability, PK, PD, and exploratory efficacy of venglustat in combination with cerezyme in adult patients with GD Type 3	10	• 208-week Three part, Open-label, Single group Assignment • Part 1: Evaluate CNS biomarkers in adult GD type 3 patients that distinguish GD3 from GD type 1, Screen adult GD3 patients who qualify for Ttmt with venglustat in Part 2, Total duration 45 days • Part 2 and 3: Safety and tolerability in GD3 patients, Total duration up to 208 weeks including : treatment of 52 weeks (Part 2) and 156 weeks (Part 3) for long term follow-up, respectively	• Primary: N of patients with AE, assessment of PD parameters (GL-1 and lyso GL1) in CSF and plasma • Secondary: PK parameters (CSF and Plasma)	• SSD: Jan. 2017 • DE (1st Part)(1): 2024

(1) Final Data Collection date for primary outcome measure

Venglustat (GCS inhibitor)
Autosomal Dominant Polycystic Kidney Disease (ADPKD) (2/3)

Study	Description	Patients	Design	Endpoints	Status
STAGED-PKD EFC15392 NCT03523728	Phase 3 Efficacy, safety, tolerability and PK of venglustat in patients at risk of rapidly progressive ADPKD	640	• Randomized, double-blind, placebo-controlled 2-stage study (18 and 24 months) • Study duration per participant is 26 months (maximal) per stage, including a screening period of 15 days, run-in period of 2 weeks, a 24-month treatment period, and a follow-up 30 days after final dose	• Primary: Rate of change in total kidney volume (TKV) based on magnetic resonance imaging (MRI) (Stage 1) and rate of change in glomerular filtration rate (eGFR) (Stage 2) Secondary: Rate of change in eGFR (Stage 1) , rate of change in TKV (Stage 2), PK assessment, safety/tolerability objectives	• SSD: Feb. 2019 • DE Stage 1: 2021 • DE Stage 2: 2023

Venglustat (GCS inhibitor)
GM2 gangliosidosis (3/3)

Study	Description	Patients	Design	Endpoints	Status
AMETHIST EFC15299 NCT04221451	Phase 3 A Multinational, Randomized, Double-blind, Placebo-controlled Study to Assess the Efficacy, Pharmacodynamics, Pharmacokinetics, and Safety of Venglustat in Late-onset GM2	62	• Multicenter, Multinational, Randomized, Double-blind, Placebo-controlled Study • The total duration is up to approximately 119 weeks, including a 60-day screening period, a 104-week treatment period, and a 6-week post-treatment safety observation period.	Primary: • Change in cerebrospinal fluid (CSF) GM2 biomarker • Change in the 9-hole pegboard test (9-HPT • Assessment of pharmacodynamic (PD) response in plasma: GL-1, GM1,GM2, GM3 biomarkers in the different subpopulations. Secondary • Safety/tolerability: Adverse events • Assessment of pharmacokinetic (PK) parameters in plasma: Cmax , tmax, AUC0-24h • Assessment of PK parameters in CSF: Cmax , tmax, AUC0-24h • Change in 25-foot walk test (FWT) • Change in Friedreich's Ataxia Rating Scale (FARS) • Change in 9-hole peg test (9-HPT)	• SSD: Q2 2020 • Primary DE: 2023

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

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

Study	Description	Patients	Design	Endpoints	Status
ELIKIDS **GD Type 1/ Type 3** EFC13738 NCT03485677	Phase 3 PK, efficacy and safety with or without Imiglucerase in pediatric patients with GD Type1/Type 3	60	• Non-randomized, open label, two cohort (with and without imiglucerase) • Cohort 1: eliglustat monotherapy • Cohort 2: eligustat plus imiglucerase	• Primary: PK (Cmax and AUC), adverse events • Secondary: changes from baseline as absolute change in g/dL for hemoglobin, % change for platelets, liver volume, and spleen volume; improvement in pulmonary disease, improvement in bone disease, thrombocytopenia, and quality of life	• SSD: Apr. 2018 • DE Primary: 2022 • DE Full completion: 2025

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

Study	Description	Patients	Design	Endpoints	Status
EXOSKEL **GD Type 1** EFC13781 NCT02536755	Phase 3 Long Term skeletal response to eliglustat in GD Type 1 adult patients who successfully completed Phase 2 or phase 3 studies	32	• Single group assignment, open label	• Primary: change from baseline in bone marrow infiltration, bone mineral density (hips and lumbar spine), skeletal imaging GD bone disease manifestations (lytic lesions, osteonecrosis, fractures and infarcts), clinical GD manifestations (mobility, bone pan, bone crisis), and bone biomarkers • Secondary: quality of life, measurement of GD Type 1 biomarkers and safety (i.e. incidence of adverse events, change from baseline in laboratory assessments (hematology), physical examinations)	• SSD: Oct. 2015 • DE Primary (2y primary outcome): 2019 • DE Full completion: 2021

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

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

Study	Description	Patients	Design	Endpoints	Status
HERA ACT 16248 / RG012-03 NCT02855268	Phase 2 Safety, Efficacy, Pharmacodynamics and Pharmacokinetics of SAR339375 (RG-012) in patients with ALPS	45	• 18-60 year old males and females with ALPS • Randomized, double-blind, placebo-control • 2 arms: SAR339375 (RG012) and placebo, in a 2:1 ratio • Duration: 48 week SC injections double-blinded treatment period. After 48 week treatment, subjects can receive a 48 week open-label extension period	• Primary: AEs; Annualized change in eGFR from baseline to 48 weeks, • Secondary: PK, Anti-drug antibodies, Percent change in eGFR values from baseline to 24 weeks and 48 weeks.	• SSD: Nov. 2017 (Restart in Nov. 2019) • DE: Apr 2022 PoC (Apr 2023 EOS)

Teriflunomide
Multiple Sclerosis (MS)

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

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 Primary: 2019 • DE Full completion for main open label period: 2021

Alemtuzumab
Relapsing Remitting Multiple Sclerosis (RRMS)

Study	Description	Patients	Design	Endpoints	Status
LemKids RRMS EFC13429 NCT03368664	Phase 3 Efficacy, Safety and Tolerability of Alemtuzumab in Pediatric Patients With Relapsing Remitting MS (RRMS) with disease activity on prior disease modifying therapy DMT	50	• In pediatric patients from 10 to <18 years of age with RRMS with disease activity on prior DMT. • Open-label, rater-blinded, single-arm, cross-over study The study will consist of different phases: • Prior DMT Phase (~4 months) – efficacy measurements on current DMT • Alemtuzumab Treatment Phase (~2 years) - The MRI based primary efficacy endpoint will be assessed over a 4 month period during this phase compared to an equal period during the prior DMT phase • Safety Monitoring Phase – safety monitoring for all patients treated with alemtuzumab (4 years post last treatment with alemtuzumab)	• Primary: The number of new or enlarging T2 lesions on brain MRI, during continuation of prior DMT (Period 1) compared to an equal period after the first course of alemtuzumab treatment (Period 2) • Secondary: The proportion of patients with new or enlarging T2 lesions , Annualized relapse rate at Year 2, Assessment of cognition test scores, Additional secondary endpoints, including PK/PD parameters and Quality of Life (QoL) measures.	• SSD: Oct. 2017 • DE: 2026

Venglustat (GCS inhibitor)
GBA related Parkinson Disease

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

Study	Description	Patients	Design	Endpoints	Status
MOVES-PD ACT14820 NCT02906020	Phase 2 Efficacy, safety, pharmacokinetics, and pharmacodynamics of venglustat (GZ402671) in patients with Parkinson's Disease (PD) carrying a glucocerebrosidase gene (GBA) mutation	245	• Patients with PD carrying a GBA mutation or other prespecified variant. • Randomized, Double-blind, Placebo Controlled, Parallel Assignment • Part 1: Increasing dose of venglustat administered once per day. Duration: up to 48 weeks outside Japan, and up to 64 weeks in Japan • Part 2: venglustat dose determined in Part 1 administered once a day Duration: 5,6-week screening, 52-week Tx period, 104-week follow-up period and 6-week post Tx observation	• Primary: Change from baseline in Movement Disorder Society Unified PD Rating Scale Part II and III score • Secondary: Change from baseline in PD Cognitive Rating Scale, Movement Disorder Society Unified PD Rating Scale Part I, II, and III score, Hoehn and Yahr score	• SSD: Dec. 2016 • DE Primary: 2021 • DE: Full completion: 2023

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

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

Study	Description	Patients	Design	Endpoints	Status
DNLI-D-0003 NCT03757351	Phase 1* Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of SAR443060(DNL747) in Subjects with Amyotrophic Lateral Sclerosis	15	• Patients with a diagnosis of laboratory-supported probable, probable or definite ALS • Randomized, Double-blind, Placebo Controlled, Cross-over Assignment • SAR443060 and placebo • 12 months open label extension	• Primary: AEs and SAEs, lab test abnormalities, clinically significant neurological abnormalities • Secondary: Pharmacokinetics, Pharmacodynamics	• SSD: Dec. 2018 • DE Primary: 2020 • DE full completion: 2021

* Phase 1 study performed by Denali

SAR442168 (BTK inhibitor)
Multiple Sclerosis (MS)

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

Study	Description	Patients	Design	Endpoints	Status
Multiple Sclerosis BEX16018 NCT04171310	Phase 1 Open-label Study of Excretion Balance and Pharmacokinetics Following a Single Oral Dose of SAR442168 in Healthy Male Subjects	6	• Healthy male subjects 30 to 65 years of age. Body Mass Index 18 up to 32 kg/m2, inclusive. Signed informed consent. Subjects must agree to the use of an adequate method of contraception for up to 3 months after discharge from the clinical unit	Primary: • To determine the excretion balance and systemic exposure of radioactivity after oral administration of [14C]-SAR442168. • To determine the pharmacokinetics of SAR442168 and its contribution to the overall exposure of radioactivity. • To collect samples in order to determine the metabolic pathways of SAR442168 and identify the chemical structures and main excretion route of the main metabolites	• SSD: 13 Nov 2019 • Completion date: Dec 2019 • DE: Dec 2020

SAR442168 (BTK inhibitor)
Multiple Sclerosis (MS)

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

Study	Description	Patients	Design	Endpoints	Status
Relapsing Multiple Sclerosis LTS16004 NCT03996291	Phase 2 Long-term Extension Safety and Efficacy Study of SAR442168 in Participants With Relapsing Multiple Sclerosis	125	Part A: Double-blind period of continued treatment with SAR442168 dose assigned in DRI15928 Part B: Open-label period of a single-group treatment with the selected Phase 3 SAR442168 dose.	Primary Objective: To determine the long-term safety and tolerability of SAR442168 in RMS participants Secondary Objective: To evaluate efficacy of SAR442168 on disease activity, assessed by clinical and imaging methods	• SSD: sept 2019 • DE: 2025

iGlarLixi (Glargine/Lixisenatide)
Type 2 Diabetes Mellitus (T2DM) (1/2)

Study	Description	Patients	Design	Endpoints	Status
Lixilan –O-AP **EFC14943** NCT03798054	Phase 3 Efficacy and Safety of iGlarLixi vs Insulin Glargine and Lixisenatide in Patients with Type 2 DM Insufficiently controlled with oral Antidiabetic Drugs	940	• Patients with T2DM diagnosed for at least 1 year, treated for at least 3 months with metformin alone or in combination with a second oral antidiabetic drug and who are not adequately controlled with this treatment, • Randomized, Parallel Group assignment, Open label, Active-controlled, • 3 arms: iGlarLixi, Lantus (insuline glargine), Lixisenatide • Study duration per patient approximately: 31 weeks: up to 6-week screening, 24-week randomized Tx and 3-day post-Tx safety follow-up	• Primary: change in HbA1c • Secondary: change in PPG, FPG, SMPG, patients with HbA1c < 7% at week 24, patients with HbA1c ≤ 6,5% at week 24, change in body weight, patients with HbA1c < 7% and no body weight gain at week 24, patients with HbA1c < 7% and no body weight gain and no documented symptomatic hypoglycemia at week 24, confirmed hypoglycemia, AEs, anti-lixisenatide antibodies.	• SSD: Feb. 2019 • DE: 2021

iGlarLixi (Glargine/Lixisenatide)
Type 2 Diabetes Mellitus (T2DM) (2/2)

Study	Description	Patients	Design	Endpoints	Status
Lixilan-L-CN **EFC14944** NCT03798080	Phase 3 Efficacy and Safety of iGlarLixi to Insulin Glargine With or Without Metformin in Patients with T2DM Insufficiently controlled on Basal insulin With or Without Oral Antidiabetic Drug(s)	426	• Patients with T2DM diagnosed for at least 1 year and treated with basal insulin for at least 6 months • Randomized, Parallel Group assignment, Open label, active-controlled • 2 arms: iGlarLixi, Lantus • Study duration per patient approximately: 33 weeks: 2-week screening, 30-week randomized Tx and 3-day post-Tx safety follow-up	• Primary: change in HbA1c • Secondary: patients with HbA1c < 7% at week 30, patients with HbA1c ≤ 6,5% at week 30, PPG, SMPG profile, patients with HbA1c < 7% and with no body weight gain, change in body weight, patients with HbA1c < 7% and with no body weight gain and no documented symptomatic hypoglycemia at week 30, patients requiring rescue therapy, FPG, confirmed hypoglycemia, AEs, anti-lixisenatide antibodies	• SSD: Feb. 2019 • DE: 2021

SAR341402 (Rapid Acting Insulin)
Type 1 Diabetes Mellitus

Study	Description	Patients	Design	Endpoints	Status
GEMELLI X **EFC15178** NCT03874715	Phase 3 Comparison of Pharmacokinetics and Immunogenicity of Alternating Use of SAR341402 to NovoLog® Versus Continuous Use of NovoLog® in Patients with T1DM also using Insulin Glargine	210	• Patients with T1DM, on continuous insulin Tx for at least 12 months prior to screening, • Randomized, Open-label, Parallel-group • 2 arms: experimental: alternative use of SAR341402 and NovoLog 4 cylces of 4 weeks each, on top of lantus; Active comparator: NovoLog for 16 weeks on top of lantus • Study duration: 18-week + 1 day, per patient: 2-week screening period, 16-week Tx period, 1-day post-Tx follow-up period.	• Primary: AUClast, AUC and Cmax of SAR341402 and NovoLog (similarity), • Secondary: Immunogenicity, hypoglycemic event, AEs, comparison of PK parameters between the two arms.	• SSD: Mar 2019 • DE: 2020

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

Study	Description	Patients	Design	Endpoints	Status
HeFH in Children and Adolescents EFC14643 NCT03510884	Phase 3 Efficacy and safety of alirocumab in children and adolescents with HeFH	150	• Patients with diagnosis of HeFH, 8 to 17 years old. Alirocumab (one of 4 doses, depending on body weight and Q2W or Q4W dose regimens) will be administered subcutaneously (SC). Patients treated with optimal dose of statin +/- other LMT(s) or non-statin LMT(s) if statin intolerant at stable dose • Randomized, double-Blind, placebo-controlled followed by an open label treatment period (2 dose tested) • Study duration: approximately 110 weeks (run-in period, if needed,: up to 4 weeks [+2 days], screening period, up to 2 weeks (+5 days), double-blind treatment period: 24 weeks, open label treatment: 80 weeks)	• Primary: % change in LDL-C from baseline to week 24 • Secondary: % change in LDL-C, % change in APO B (Apo B), % change in non-high density LP cholesterol (non HDL-C), % change in Total-C, patients with LDL-C level lower than 130 mg/dL (3.37 mmol/L), patients with LDL-C level lower than 110 mg/dL (2.84 mmol//L), % change in Lp(a), in HDL-C, in TG and in ApoA-1. Number of AE, maturing cognition (Cogstate battery test) and pubertal development (Tanner stage)	• SSD: May 2018 • DE: 2022

Alirocumab (anti-PCSK-9 mAb)
Neurocognitive Evaluation

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

Fitusiran (siRNA targeting Antithrombin/AT3)
Hemophilia A & B (1/6)

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

Study	Description	Patients	Design	Endpoints	Status
Hemophilia A or B LTE14762 ALN- AT3SC-002 NCT02554773	Phase 1/2 Hemophilia A Hemophilia B Long term Safety and Efficacy of Fitusiran in patients with moderate or severe Hemophilia A or B, who have previously participated in ALN-AT3SC-001	34	• In male patients (≥ 18 years old) • Single Group assignment, Open-label • Subjects are administered SC fitusiran once every month for approximately 6 years.	• Primary: incidence of treatment-emergent AEs, SAEs, and AEs leading to study drug discontinuation Secondary: Annualized bleed rate, time intervals between bleeding episodes, Weight-adjusted consumption of FVIII, FIX, or BPA, QOL assessed by an EQ-5D questionnaire and HAEM-A-QoL, Antithrombin levels, Thrombin Generation levels	• SSD: Sep. 2015 • DE: 2024

Fitusiran (siRNA targeting Antithrombin/AT3)
Hemophilia A & B (2/6)

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

Study	Description	Patients	Design	Endpoints	Status
ATLAS-INH **EFC14768** **ALN- AT3SC-003** **NCT03417102**	Phase 3 Hemophilia A Hemophilia B Efficacy and Safety of Fitusiran in patients with Hemophilia A or B, with Inhibitory Antibodies to Factor VIII or IX, who are not receiving prophylactic treatment	54	• In patients (Males ≥ 12 years old) • Randomized in a 2:1 ratio - Patients randomized to the fitusiran treatment arm will receive open label fitusiran as an SC injection once monthly, for a total of 9 months - Patients in on-demand arm will receive on-demand BPA therapy per Investigator discretion to treat bleeding episodes	• Primary: Annualized Bleeding Rate (ABR) in the efficacy period • Secondary: ABR in the treatment period, Annualized spontaneous bleeding rate in the efficacy period, Annualized joint bleeding rate in the efficacy period, Change in HAEM-A-QOL score in the treatment period, ABR in the onset period	• SSD: Mar. 2018 • DE: 2020

Fitusiran (siRNA targeting Antithrombin/AT3)
Hemophilia A & B (3/6)

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

Study	Description	Patients	Design	Endpoints	Status
ATLAS-A/B **EFC14769** **ALN- AT3SC-004** **NCT03417245**	Phase 3 Hemophilia A Hemophilia B Efficacy and Safety of Fitusiran in patients with Hemophilia A or B, without Inhibitory Antibodies to Factor VIII or IX, who are not receiving prophylactic treatment	120	• In patients (Males ≥ 12 years old), • Randomized in a 2:1 ratio: - Patients randomized to the fitusiran treatment arm will receive open-label fitusiran once monthly for a total of 9 months; - Patients in the on-demand arm will receive on-demand factor concentrate therapy per Investigator discretion to treat bleeding episodes	• Primary: Annualized Bleeding Rate (ABR) in the efficacy period • Secondary: ABR in the treatment period, Annualized spontaneous bleeding rate in the efficacy period, Annualized joint bleeding rate in the efficacy period, Change in HAEM-A-QOL score in the treatment period, ABR in the onset period	• SSD: Jul. 2018 • DE: 2021

Fitusiran (siRNA targeting Antithrombin/AT3)
Hemophilia A & B (4/6)

Study	Description	Patients	Design	Endpoints	Status
ATLAS-PPX **EFC15110** **ALN- AT3SC-009** **NCT03549871**	Phase 3 Hemophilia A Hemophilia B Switching study to describe the Efficacy and safety of Fitusiran prophylaxis in Patients with Hemophilia A or B, with or without inhibitory antibodies to factor VIII (FVIII) or factor IX, and previously receiving Factor or Bypassing Agent Prophylaxis	70	• In patients (males ≥ 12 years old), • Single Group assignment, Open-label • The study has 3 periods: - 6-Month factor/bypassing agent prophylaxis period in which patients will continue their pre study, regularly scheduled prophylaxis regimen with factor concentrates or bypassing agents - 1-Month onset period in which patients receive their first dose of fitusiran while continuing their factor/bypassing agent prophylaxis for up to 14 days - 6-Month fitusiran efficacy period in which patients receive fitusiran as a once monthly prophylaxis	• Primary: annualized bleeding rate (ABR) in the fitusiran efficacy period and the factor or BPA in prophylaxis period • Secondary: annualized spontaneous bleeding rate and annualized joint bleed rate in the fitusiran efficacy period and the factor or BPA in prophylaxis period, Quality of Life (QOL) measured by Haem-A-QOL Questionnaire, ABR in the fitusiran onset period (1 month), ABR in the fitusiran Tx period (7 months)	• SSD: Sept 2018 • DE: 2021

Fitusiran (siRNA targeting Antithrombin/AT3)
Hemophilia A & B (5/6)

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

Study	Description	Patients	Design	Endpoints	Status
ATLAS-OLE **LTE15174** **ALN-AT3SC-005** **NCT03754790**	Phase 3 Hemophilia A Hemophilia B Long-term Safety and Efficacy of Fitusiran in Patients with Hemophilia A or B With or Without Inhibitory Antibodies to Factor VIII or X , who have previously participated in any of the phases 3 studies with fitusiran	244	• In patients (≥ 12 years old), • Single Group assignment, Open-label • Study duration: the study consists in screening period up to 30 days, a 48-month open label Tx period and a follow-up period up to 6 months after the last dose of fitusiran.	• Primary: Incidence, severity, relatedness, and seriousness of AEs, and laboratory assessments, • Secondary: annualized bleeding rate (ABR), annualized spontaneous bleeding rate and annualized joint bleed rate in the Tx period, Quality of Life (QOL) measured by HAEM-A-QOL Questionnaire	• SSD: Jan. 2019 • DE: 2025

Fitusiran (siRNA targeting Antithrombin/AT3) Hemophilia A & B (Pediatric) (6/6)

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

Study	Description	Patients	Design	Endpoints	Status
ATLAS-PEDS **Hemophilia A** **Hemophilia B** **NCT03974113**	Phase 3 An Open-label, Multinational Study of Fitusiran Prophylaxis in Male Pediatric Subjects Aged 1 to Less Than 12 Years With Hemophilia A or B	12	• Male, aged 1 to <12 years, • Single Group assignment, Open-label • Study duration per participant is approximately 160 weeks, including a 12-week fitusiran efficacy period	• Primary: Lowering of plasma antithrombin (AT) activity level [Time Frame: Day 1 to Day 85] • Secondary: Number of participants reported with adverse events , pharmacokinetics (PK): Cmax, Tmax, Ctrough)	• SSD: June 2019 • DE: 2024

Sutimlimab (BIVV009 - Anti Complement C1s mAb)
Complement Mediated Disorders

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

Study	Description	Patients	Design	Endpoints	Status
BIVV009-01 NCT02502903	Phase 1 Safety, Tolerability and Activity of BIVV009 in Healthy Volunteers and Patients with Complement-Mediated Disorders	122	• Healthy male and female volunteers, • Randomized, Double-blind, Parallel assignment, • Part A : single ascending dose (7 BIVV009 dose levels) or placebo • Part B: Multiple ascending dose (2 BIVV009 dose levels) or placebo, • Part C: Multiple dose in a single cohort of patients with various complement-mediated disorders, • Part E: Multiple dose in a single cohort of patients with CAD previously treated by BIVV009.	• Primary: AEs, • Secondary: PK, classical pathway complement system activity, complement system-related biomarkers, coagulation system-related biomarkers, disease-related biomarkers.	• SSD: 2015 • DE: 2021

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

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

Study	Description	Patients	Design	Endpoints	Status
TNT009-201/ BIVVOO9-201 TDR16218 NCT03275454	Phase 1 Safety, PK and PD of BIVV009 in patients with Chronic Immune Thrombocytopenia (ITP)	16	• Patients suffering from chronic ITP. Open-label, Single Group assignment • Part A: Bi-weekly IV infusion of BIVV009 up to 21 weeks • Part B: long-term treatment period (for 52 weeks) for patients who have had benefit from BIVV009 during Part A; patients undergo monitored washout from BIVV009 at end of Part A and enter Part B upon return of thrombocytopenia.	• Primary: TEAEs, premature study terminations, Clinical Laboratory Abnormalities • Efficacy endpoints: Part A & B: Change in platelet count; independence from additional ITP therapy; Number of patient who achieve complete response (CR), response (R); Duration of CR and R; Time to increased platelet count > 30, 50, and 100 x 10^9/L; number of patients with loss of CR, loss of R, • PK/PD endpoints: PK parameters, anti-drug antibodies, PD measures (Complement factor measures, thrombopoietin levels , immature platelet fraction, platelet autoantibody/autoantigen)	• SSD: Aug. 2017 • DE: 2022

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

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

Study	Description	Patients	Design	Endpoints	Status
Cardinal **BIVV009-03** NCT03347396	Phase 3 Efficacy and Safety of BIVV009 in patients with Primary Cold Agglutinin Disease with a recent history of Blood Transfusion	24	• Patients suffering from primary cold agglutinin disease (CAD) with at least one blood transfusion within 6 months of enrollment • Open-label, Single Group assignment • Part A (required for registration): biweekly IV infusion of BIVV009 up to week 26 • Part B: long-term safety and durability of response extension phase for patients having completed Part A, BIVV009 dosing for up to 1 year after Part A LPO	• Primary (Part A): response rate (≥ 2g/dl increase in Hgb OR Hgb >12g/dl AND no transfusion required); • Secondary (Part A): change in bilirubin, change in FACIT-Fatigue Scale Score, change in LDH, number of transfusions and blood units and change in Hgb; • Part B: TEAEs, hemoglobin, bilirubin, FACIT-F, LDH, transfusion, haptoglobin, HRU.	• SSD: Nov. 2017 • DE: Part A: 2019, Part B: 2021

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

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

ST400 (gene-editing technology)
Beta-thalassemia

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

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

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

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

Study	Description	Patients	Design	Endpoints	Status
PRECIZN-1 **003SCD101** **NCT03653247**	Phase 1/2 Safety, Tolerability and Efficacy of BIVV003 for Autologous Hematopoietic Stem Cell Transplantation in Patients With severe Sickle Cell Disease	8	• Patients suffering from severe SCD • Open-Label, Single Group Assignment, single dose	• Primary: % of patients alive post-transplantation at D100, at week 52, at week 104, % of patients with successful engraftment, AEs, SAEs, • Secondary: CD34+HSPC yield from Plerixafor stem cell mobilization, % of patients with sufficient stem cell mobilization, yield of ZFN-edited IP, time to initial neutrophil recovery, time to platelet recovery, % of patients with maintenance of absolute neutrophil count ≥ 500/mcL, % of patients with maintenance of platelets count ≥ 50 000/mcL, change from baseline in HbF, in %F, in HbS, in REC, in LDH, in haptoglobin and bilirubin, QoL	• SSD: Jan. 2019 • DE: 2023

Caplacizumab - Cablivi™
Acquired Thrombotic Thrombocytopenic Purpura

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

Study	Description	Patients	Design	Endpoints	Status
Post-HERCULES **ALX0681-C302** **NCT02878603**	Phase 3 Evaluate the long-term safety and efficacy of caplacizumab, evaluate safety and efficacy of repeated use of caplacizumab and characterize the long-term impact of acquired Thrombotic Thrombocytopenic Purpura (aTTP).	104	• Prospective follow-up for adult patients (18 years and older) with acquired TTP who completed HERCULES • Single group assignment, open label • Study duration: Initial IV loading dose, followed by daily SC caplacizumab injections for the duration of daily PEX and 30 days thereafter. Treatment may be extended for a maximum of 4 weeks.	• Primary: proportion of subjects with TTP-related events, # of and time to TTP-related events, mortality rate, proportion of subjects with, # of and time to recurrence of disease, proportion of subjects with reported major thromboembolic events, # of and time to major thromboembolic events, cognitive function, quality of life assessment and immunogenicity.	• SSD: . Oct 2016 • DE: 2021

BIVV020 Complement C1S inhibitor
Cold Agglutinin Disease

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

Study	Description	Patients	Design	Endpoints	Status
PDY16370 **NCT04269551**	Phase 1b A Multicenter, Phase 1b, Open Label, Nonrandomized, Single Dose Study Evaluating the Safety, Tolerability and Activity of BIVV020 in Adults With Cold Agglutinin Disease	18	• Single group assignment, open label • Up to 23 weeks (screening period up to 8 weeks, treatment period 15 weeks).	Primary : To assess the safety and tolerability, after a single dose of intravenous (IV) BIVV020 Secondary : To assess • The effect of BIVV020 on complement mediated hemolysis • The pharmacodynamics (PD) of BIVV020 relating to complement inhibition • The pharmacokinetics (PK) of BIVV020 • The immunogenicity of BIVV020	• SSD: Jul 2020 • DE: 2021

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

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

Dengue Vaccine
Different schedules
Asia Pacific, Latin America Regions

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

Dengue Vaccine
Co-administration w/ HPV
Latin America Region

Study	Description	Patients	Design	Endpoints	Status
NCT02979535	Phase 3b Immunogenicity and Safety of a Tetravalent Dengue Vaccine Administered Concomitantly or Sequentially With Cervarix®	480	• Randomized, open-label, multicenter study	• Immunogenicity and safety of a Tetravalent Dengue Vaccine administered concomitantly or sequentially with Cervarix®	• SSD: Nov. 2016 • DE: 2020

Dengue Vaccine
Co-administration w/ HPV
Asia Pacific Region

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

Dengue Vaccine
Latin America, Asia Pacific Regions

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

AcP Primary Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT00855855	Phase 4 Surveillance Program to Determine Product Specific Rates of Invasive Hib Disease	510 000	• Observational	• Surveillance for Hib disease.	• SSD: Feb. 2009 • DE: 2020

New Pertussis Vaccine
Latin America Region

Study	Description	Patients	Design	Endpoints	Status
NCT03147898	Phase Epidemiology Observational Study Describing the Immune Profile Induced By Pertussis Vaccines	90	• Observational, multicenter trial	• Immune response to booster dose	• SSD: Apr. 2017 • DE: 2020

Flu seasonal Vaccine
North America Region

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

Meninge Vaccine
MenQuadfi - Co administration
North America Region

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

Study	Description	Patients	Design	Endpoints	Status
NCT03537508	Phase 3 Safety and Immunogenicity for Infants, with co administration with routine pediatric vaccines	2475	Modified double blind study, randomized, parallel groups, active controlled, multicenter	• Immunogenicity and safety	• SSD: Apr. 2018 • DE: 2023

Meninge Vaccine
MenQuadfi - Alternative schedules
Greater Europe Region

Study	Description	Patients	Design	Endpoints	Status
NCT03547271	Phase 3 Safety and ImmunogenIcity for alternative schedules in infants	1540	• Partially modified double blind, randomized, parallel group, active controlled, multi center	• immunogenicity and safety	• SSD: Dec. 2018 • DE: 2023

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

Study	Description	Patients	Design	Endpoints	Status
NCT03630705	Phase 3 Safety and immunogenicity 3 dose schedule Quadrivalent Meningococcal conjugate vaccine	825	• Interventional, randomized, parallel assignement, active controlled multi center study	• Immunogenicity and safety	• SSD: Oct. 2018 • DE: 2023

Meninge Vaccine
MenQuadfi
Latin America, North America

Study	Description	Patients	Design	Endpoints	Status
NCT03691610	Phase 3 Safety & Immunogenicity 2-dose Trial in Toddlers	940	• Interventional, modified double blind, Randomized, parrallel assignement active controlled multi center study.	• Immunogenicity and safety	• SSD: Oct. 2018 • DE: 2022

Flu QIV HD Vaccine
North America Region

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

Rabies Vaccine
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT03700242	Phase3 Immunogenicity and safety of HDCV with abbreviated pre-exposure regimens Trial	570	• Interventional, Randomized, Parallel Assignment,	• Immunogenicity and safety	• SSD: Sep. 2018 • DE: 2021

Meninge Vaccine
MenQuadfi Men C
Greater Europe Region

Study	Description	Patients	Design	Endpoints	Status
NCT03890367	Phase 3 Immunogenicity and Safety of Quadrivalent Meningococcal Conjugate Vaccine Compared With Two Meningococcal Reference Vaccines in Europeans Toddlers	675	• Randomized, parallel assignment, modified double-blind (triple masking - Participant, Investigator, Outcomes Assessor) conducted in Denmark, Finland, and Germany.	• Immunogenicity and Safety Assessment.	• SSD: Sep.2019 • DE: 2021

Meninge Vaccine
MenQuadfi
Africa and Middle-East Region

Study	Description	Patients	Design	Endpoints	Status
NCT03869866	Phase 3 Immunogenicity and Safety of a Quadrivalent Meningococcal Conjugate Vaccine in Potential Pilgrims Aged 56 Years and Older in Turkey	330	• Interventional, single group assignment, open label conducted in Turkey.	• Immunogenicity and Safety Assessment.	• SSD: Apr.2019 • DE: 2021

Rabies Vaccine
VRVg
Greater Europe Region

Study	Description	Patients	Design	Endpoints	Status
NCT03965962	Phase 3 Purified Vero Rabies Vaccine Compared With Two Reference Rabies Vaccines in a Simulated Post-Exposure Regimen in Adults	504	• Interventional, randomized, parallel assignment: three modified double-blind groups + one open label group.	• Immunogenicity and Safety Assessment.	• SSD: Jun.2019 • DE: 2021

New Pertussis Vaccine
North American Region

Study	Description	Patients	Design	Endpoints	Status
NCT03958799	Phase 1 Describe the Safety Profile and Compare the Immune Response of 4 Different Formulations of an Investigational Tdap Vaccine When Compared to Licensed Tdap Vaccine in Young Adults in Canada	90	• Interventional, randomized, parallel assignment, modified double-blind.	• Immunogenicity and Safety Assessment.	• SSD: Jun.2019 • DE: 2022

Meninge Vaccine MenQuadfi
North American Region

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

Study	Description	Patients	Design	Endpoints	Status
NCT04084769	Phase 3b Evaluate the Immune Response After a Booster Dose of a Quadrivalent Meningococcal Conjugate Vaccine When Administered Alone or Concomitantly With a Licensed Meningococcal Serogroup B Vaccine,in Participants Who Received Primary Quadrivalent Meningococcal Conjugate Vaccine (MCV4)	600	• Interventional, randomized, parallel assignment, open label.	• Immunogenicity Assessment.	• SSD: Sep.2019 • DE: 2021

Flu QIV HD Vaccine
Greater Europe

Study	Description	Patients	Design	Endpoints	Status
NCT04024228	Phase 3 Assess the Immune Response and the Safety Profile of a High-Dose Quadrivalent Influenza Vaccine (QIV-HD) Compared to a Standard-Dose Quadrivalent Influenza Vaccine (QIV-SD) in Europeans Adults 60 Years of Age and Older	1540	• Interventional, randomized, parallel assignment, modified double-blind.	• Immunogenicity and Safety Assessment.	• SSD: Oct.2019 • DE: 2020

Flu QIV SHZ - CN
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT04210349	Phase 3 Assess the Immune Response and the Safety Profile of the Shenzhen Quadrivalent Inactivated Influenza Vaccine Versus the Shenzhen Trivalent Inactivated Influenza Vaccine in Chinese Subjects From 6 Months of Age	6134	• Interventional, randomized, parallel assignment, open-label in step 1 and modified double-blind n step 2.	• Immunogenicity and Safety Assessment.	• SSD: Jan.2020 • DE: 2021

Next Gen Flu
North America Region

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

Study	Description	Patients	Design	Endpoints	Status
NCT04144179	Phase 1 Assess the Immune Response and the Safety Profile of Quadrivalent Recombinant Influenza Vaccine Formulations Containing Different H3 Hemagglutinin Antigens in Healthy Adult Subjects 18 to 30 Years of Age	150	• Interventional, randomized, parallel assignment, open label.	• Immunogenicity and Safety Assessment.	• SSD: Nov.2019 • DE: 2021

Meninge Vaccine
MenQuadfi - Booster
Africa and Middle East Regions

Study	Description	Patients	Design	Endpoints	Status
NCT04143061	Phase 3 Assess the Immune Response and the Safety Profile of an Investigational Quadrivalent Meningococcal Conjugate Vaccine in Healthy Adults, Adolescents, and Children in India and Healthy Adolescents and Children in the Republic of South Africa	1332	• Interventional, randomized, parallel assignment, modified double-blind.	• Immunogenicity and Safety Assessment.	• SSD: Dec.2019 • DE: 2022

vYF Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT04142086	Phase 1 Assess the Immune Response , Tolerability, and the Safety Profile of a Investigational Yellow Fever Vacine (vYF) Candidate Vaccine in Adults	572	• Interventional, randomized, parallel assignment, observer-blind.	• Immunogenicity, Tolerability, and Safety Assessment.	• SSD: Jan 2020 • DE: 2021

Meninge Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT04142242	Phase 3 Assess the Immune Response and the Safety Profile of a Single Dose of MenACYW Conjugate Vaccine at Least 3 Years Following Initial Vaccination With Either Menomune® Vaccine or MenACYW Conjugate Vaccine in Older Adults	560	• Interventional, randomized, parallel assignment, open label.	• Immunogenicity and Safety Assessment.	• SSD: Oct.2019 • DE: 2022

Rabies Vaccine
VRVg
Asia Pacific Region

Study	Description	Patients	Design	Endpoints	Status
NCT04127786	Phase 3 Assess the Immune Response and the Safety Profile of a Purified Vero Rabies Vaccine - Serum Free in Comparison With Verorab® and Imovax® Rabies, in a Pre-exposure Regimen in Both Pediatric and Adult Populations and a Single Booster Dose of Purified Vero Rabies Vaccine - Serum Free Administered at 1 Year Post Primary Series in a Subset of Adults in Thailand	1010	• Interventional, randomized, parallel assignment, observer blind.	• Immunogenicity and Safety Assessment.	• SSD: Oct.2019 • DE: 2021

Flu QIV HD Vaccine
Greater Europe

Study	Description	Patients	Design	Endpoints	Status
NCT04137887	Phase 3 Assess the Relative Effectiveness of a High-Dose Quadrivalent Influenza Vaccine Versus a Standard-Dose Quadrivalent Influenza Vaccine in Subjects 65 Years of Age and Older	68000	• Interventional, randomized, parallel assignment, modified double-blind.	• Effectiveness Assessment.	• SSD: Nov.2019 • DE: 2022

HSV Vaccine
North America Region

Study	Description	Patients	Design	Endpoints	Status
NCT04222985	Phase 1 / 2 Assess the Safety and Efficacy of 4 Investigational HSV 2 Vaccines in Adults With Recurrent Genital Herpes Caused by HSV 2	381	• Interventional, randomized, sequential assignment, quadruple masking.	• Safety & Effectiveness Assessment.	• SSD: Feb.2020 • DE: 2023

Adacel Quadra Vaccine
Africa and Middle East Regions

Study	Description	Patients	Design	Endpoints	Status
NCT04300192	Phase 4 Evaluate the Immune Response After Booster Vaccination With Tdap-IPV Vaccine (Against Tetanus, Diphtheria, Pertussis and Poliomyelitis) in Children 9-13 Years Who Received Different Pertussis Primary Vaccine Regimens in Republic of South Africa	350	• Interventional, randomized, parallel assignment, open label, prevention.	• Immunogenicity and Safety Assessment.	• SSD: May.2020 • DE: 2022