

Roche Group development pipeline

Marketed products development programmes

Roche Pharma global development programmes

Roche Pharma research and early development (pRED)

Genentech research and early development (gRED)

Spark

Changes to the development pipeline

Q2 2023 update

New to phase I	New to phase II	New to phase III	New to registration
4 NMEs: RG6449 HBsAg MAb – chronic hepatitis B RG6353 HLA-G CD3 TCB – solid tumors RG6537 AR degrader – mCRPC CHU SAIL66 – solid tumors	1 NME: RG6536 vixarelimab – IPF & SSc-ILD 1 AI: RG6171 giredestrant – endometrial cancer	1 AI: RG6114 inavolisib – post CDKi HR+ BC	1 NME (US & EU) RG6107 crovalimab – PNH* 1 AI (US): RG7716 Vabysmo – BRVO/CRVO
Removed from phase I	Removed from phase II	Removed from phase III	Approvals
4 NMEs: RG6007 HLA-A2-WT1 x CD3 – AML RG7637 – psychiatric disorders RG6392 – oncology SQZ PBMC vaccine – solid tumors	1 NME: RG6358 SPK-8016 – hemophilia A with inhibitors to factor VIII	1 AI: RG3625 TNKase – stroke	1 NME (US & EU): RG6026 Columvi (glofitamab) – 3L+ DLBCL

Roche Group development pipeline



Phase I (49 NMEs + 12 AIs)

RG6026	Columvi (glofitamab) monotherapy + combos	heme tumors
RG6058	tiragolumab combos	heme & solid tumors
RG6076	englumafusp alfa (CD19-4-1BBL) combos	heme tumors
RG6114	inavolisib	solid tumors
RG6156	EGFRvIII x CD3	glioblastoma
RG6160	cevestamab	r/r multiple myeloma
RG6171	giredestrant monotherapy + combos	solid tumors
RG6180	autogene cevumeran ± T	solid tumors
RG6185	belvarafenib + Cotellic ± T	solid tumors
RG6189	FAP-CD40 ± T	solid tumors
RG6194	runimotamab	BC
RG6234	forimtamig	multiple myeloma
RG6264	Phesgo OBI	HER2+ BC
RG6279	eciskafusp alfa (PD1-IL2v) ± T	solid tumors
RG6286	-	colorectal cancer
RG6292	CD25 MAb combos	solid tumors
RG6323	IL15/IL15Ra-Fc ± T	solid tumors
RG6330	divarasib (KRAS G12C) monotherapy + combos	solid tumors
RG6333	CD19 x CD28 + Columvi (glofitamab)	r/r NHL
RG6344	BRAF inhibitor (3)	solid tumors
RG6353	HLA-G CD3 TCB	solid tumors
RG6411	-	solid tumors
RG6433	SHP2i combos	solid tumors
RG6440	Anti-latent TGF-β1 (SOF10)	solid tumors
RG6512	FIXa x FX	hemophilia
RG6524	DLL3 trispecific	solid tumors
RG6526 ¹	camonsertib	solid tumors
RG6537	AR degrader	mCRPC
RG6538 ²	P-BCMA-ALLO1	multiple myeloma
RG7446	Morpheus platform	solid tumors
RG7601	Venclexta ± azacitidine	r/r MDS

RG7802	cibisatamab ± T	solid tumors
RG7827	FAP-4-1BBL monotherapy + combos	solid tumors
RG7828	Lunsumio monotherapy + combos	heme tumors
CHU	glypican-3 x CD3	solid tumors
CHU	codrituzumab	HCC
CHU	CD137 switch antibody	solid tumors
CHU	RAS inhibitor	solid tumors
CHU	SPYK04	solid tumors
CHU	SAIL66	CLDN6+ solid tumors
RG6107	crovalimab	lupus nephritis
RG6287	-	aGVHD
RG6315	-	immunologic disorders
RG6421	TMEM16A potentiator	cystic fibrosis
RG7828	Lunsumio	SLE
CHU	anti-HLA-DQ2.5 x gluten peptides	celiac disease
CHU	RAY121	immunology
RG6006	zosurabalpin (Abx MCP)	bacterial infections
RG6319	LepB inhibitor	complicated urinary tract infection
RG6449	HBsAg MAb	chronic hepatitis B
RG6035	BS-CD20 MAb	multiple sclerosis
RG6091	rugonersen	Angelman syndrome
RG6163	-	psychiatric disorders
RG6182	MAGLi	multiple sclerosis
RG6289	-	Alzheimer's
RG6418*	selnoflast	inflammation
RG6120	zifibancimig (VEGF-Ang2 DutaFab)	nAMD
RG6209	-	retinal disease
RG6351	-	retinal disease
RG7921	-	RVO
CHU	anti-IL-8 recycling antibody	endometriosis

	New Molecular Entity (NME)
	Additional Indication (AI)
	Oncology / Hematology
	Immunology
	Infectious Diseases

	Metabolism
	Neuroscience
	Ophthalmology
	Other

Phase II (23 NMEs + 10 AIs)

RG6026	Columvi (glofitamab) + chemo	1L ctDNA high risk DLBCL
	tiragolumab + T	NSCLC
RG6058	tiragolumab + T + chemo	NSCLC neoadj-adj
	tiragolumab + T	cervical cancer
	tiragolumab + T	1L PD-L1+ mSCCHN
RG6107	crovalimab	sickle cell disease
RG6139	tobemstomig (PD1 x LAG3)	solid tumors
RG6171	giredestrant	endometrial cancer
RG6180	autogene cevumeran + pembrolizumab	1L melanoma
RG6357	dirloctogene samoparvovec (SPK-8011)	hemophilia A
RG6299 ³	ASO factor B	IgA nephropathy
RG6341	-	chronic cough
RG6536	vixarelimab	IPF/SSc-ILD
RG7854/ RG6346/ RG6084**	ruzotolimod/xalnesiran/PDL1 LNA	HBV
RG6359	SPK-3006	Pompe disease
RG1662	basmisanol	Dup15q syndrome
RG6042	tominersen	Huntington's
RG6100	seminomab	Alzheimer's
RG6102	trontinemab	Alzheimer's
RG6237	latent myostatin + Evrysdi	SMA
	latent myostatin	FSHD
RG6416	bepranemab	Alzheimer's
RG7314	balovaptan	post-traumatic stress disorder
RG7412	crenezumab	familial Alzheimer's healthy pts
RG7816	alogabat	ASD
RG7906	ralmitaront	schizophrenia
RG7935	prasinezumab	Parkinson's
RG6179	anti-IL-6	DME
RG6299 ³	ASO factor B	geographic atrophy
RG6501	OpRegen	geographic atrophy
RG7774	vicasinabin	DR

RG-No - Roche/Genentech; CHU - Chugai managed; ¹Repare Therapeutics managed; ²Poseida Therapeutics managed; ³IONIS managed; T=Tecentriq; BS=Brain Shuttle; OBI=On-Body Delivery System; *also developed in Immunology; **combination platform

Status as of July 27, 2023

Roche Group development pipeline

Phase III (8 NMEs + 38 AIs)

RG3502	Kadcyla + T	HER-2+ eBC high-risk	RG3648	Xolair	food allergy
RG6026	Columvi (glofitamab) + chemo	2L+ DLBCL	RG6149	astegolimab	COPD
RG6058	tiragolumab + T	1L PD-L1 high NSCLC	RG7159	Gazyva	lupus nephritis
	tiragolumab + T	1L esophageal cancer		Gazyva	membranous nephropathy
	tiragolumab + T	locally advanced esophageal cancer		Gazyva	systemic lupus erythematosus
	tiragolumab + T	stage III unresectable 1L NSCLC		Gazyva	childhood onset idiopathic nephrotic syndrome**
	tiragolumab + T	1L non-squamous NSCLC		Gazyva	childhood onset idiopathic nephrotic syndrome**
RG6107	crovalimab	aHUS	RG6152	Xofluza	influenza, pediatric (0-1 year)
RG6114	inavolisib	1L HR+ mBC	RG1594	Xofluza	influenza direct transmission
	inavolisib	post CDKi HR+ BC		Ocrevus higher dose	RMS & PPMS
RG6171	giredestrant	1L ER+/HER2- mBC	RG6168	Ocrevus SC	RMS & PPMS
	giredestrant	ER+ BC adj		Enspryng	myasthenia gravis
	giredestrant + Phesgo	1L ER+/HER2+ BC		Enspryng	MOG-AD
RG6330	divarasib (KRAS G12C)	2L NSCLC	RG6356	Enspryng	autoimmune encephalitis
RG7446	Tecentriq + platinum chemo	NSCLC periadj	RG7845	Elevidys (delandistrogene moxeparvovec)	DMD
	Tecentriq	NMIBC, high-risk	RG6179	fenebrutinib	RMS
	Tecentriq ± chemo	SCCHN adj		fenebrutinib	PPMS
	Tecentriq + capecitabine or carbo/gem	1L TNBC	RG6321	anti-IL-6	UME
	Tecentriq + Avastin	HCC adj		Susvimo	DME
	Tecentriq	ctDNA+ high-risk MIBC		Susvimo	DR
	Tecentriq + lurbinectedin	1L maintenance SCLC		Susvimo	wAMD, 36-week
RG7601	Venclexta	r/r MM t(11:14)	New Molecular Entity (NME) Additional Indication (AI) Oncology / Hematology Immunology Infectious Diseases Metabolism Neuroscience Ophthalmology Other		
	Venclexta + azacitidine	1L MDS			
RG7828	Lunsumio + lenalidomide	2L+ FL			
	Lunsumio + Polivy	2L+ DLBCL			
RG7853	Alecensa	ALK+ NSCLC adj			

Registration US & EU (1 NME + 4 AIs)

RG6107*	crovalimab ¹	PNH
RG7446	Tecentriq SC	all approved indications
RG7916	Evrysdi ²	SMA pediatric <2months
RG7716	Vabysmo ³	BRVO
	Vabysmo ³	CRVO

¹US filing acceptance pending

²Approved in US, filed in EU

³Filed in US

T=Tecentriq

*First filed in China in Q3 2022

**also known as pediatric nephrotic syndrome (PNS)

	New Molecular Entity (NME)		Metabolism
	Additional Indication (AI)		Neuroscience
	Oncology / Hematology		Ophthalmology
	Immunology		Other
	Infectious Diseases		

¹IONIS managed

Status as of July 27, 2023

✓ Indicates submission to health authorities has occurred
Unless stated otherwise submissions are planned to occur in US and EU
OBI=On-Body Delivery System, ¹filing timeline based on data from interim analysis
*also known as pediatric nephrotic syndrome (PNS)

Status as of July 27, 2023

Major pending approvals 2023



US		EU		China		Japan-Chugai	
RG7446	Tecentriq SC all approved indications Filed Nov 2022	RG7916	Evrysdi SMA presymptomatic pediatric <2mo Filed Nov 2021	RG6264	Phesgo HER-2+ BC Filed July 2022	RG6264	Phesgo HER-2+ BC/CC Filed Sept 2022
RG7716	Vabysmo BRVO/CRVO Filed May 2023	RG1569	Actemra SS-ILD Filed Aug 2022	RG6107	crovalimab PNH Filed Aug 2022	RG1569	Actemra Cytokine release syndrome (CRS) Filed Feb 2023
RG6107*	crovalimab PNH Filed June 2023	RG7446	Tecentriq SC all approved indications Filed Nov 2022	RG6026	Columvi (glofitamab) 3L+ DLBCL Filed Dec 2022	RG7716	Vabysmo BRVO/CRVO Filed April 2023
		RG6107	crovalimab PNH Filed June 2023	RG7716	Vabysmo nAMD/DME Filed June 2023	RG6107	crovalimab PNH Filed June 2023

Status as of July 27, 2023

	New Molecular Entity (NME)
	Additional Indication (AI)
	Oncology / Hematology
	Immunology
	Infectious Diseases

	Metabolism
	Neuroscience
	Ophthalmology
	Other

SC=Subcutaneous
*US filing acceptance pending

Major granted approvals 2023



US		EU		China		Japan-Chugai	
RG7596	Polivy 1L DLBCL (US) April 2023	RG6152	Xofluza influenza pediatric Jan 2023	RG7596	Polivy 1L DLBCL Jan 2023		
RG6026	Columvi (glofitamab) 3L+ DLBCL June 2023	RG6013	Hemlibra moderate hemophilia A Jan 2023	RG7596	Polivy r/r DLBCL Jan 2023		
		RG6413+ RG6412	Ronapreve* SARS-CoV-2 hospitalized May 2023	RG6152	Xofluza influenza pediatric 5 to <12 years March 2023		
		RG6026	Columvi (glofitamab) 3L+ DLBCL July 2023	RG7916	Evrysdi SMA presymptomatic pediatric <2mo June 2023		

Status as of July 27, 2023

	New Molecular Entity (NME)		Metabolism
	Additional Indication (AI)		Neuroscience
	Oncology / Hematology		Ophthalmology
	Immunology		Other
	Infectious Diseases		

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Hemlibra (emicizumab, RG6013)

Factor VIII mimetic for treatment of hemophilia A

Indication	Hemophilia A patients without inhibitors to factor VIII	Hemophilia A patients with and without inhibitors to Factor VIII, dosing every 4 weeks
Phase/study	Phase III HAVEN 3	Phase III HAVEN 4
# of patients	N=135	N=46
Design	Patients on FVIII episodic treatment prior to study entry: ARM A: Hemlibra prophylaxis qw ARM B: Hemlibra prophylaxis q2w ARM C: Episodic FVIII treatment; switch to Hemlibra prophylaxis possible after 24 weeks Patients on FVIII prophylaxis prior to study entry: ARM D: Hemlibra prophylaxis qw	Part I: Pharmacokinetic run-in part (N=6); Hemlibra q4w Part II: Expansion part (N=40); Hemlibra q4w
Primary endpoint	Number of bleeds over 24 weeks	Number of bleeds over 24 weeks
Status	- Study met primary and key secondary endpoints Q4 2017 - FDA granted Breakthrough Therapy Designation April 2018 - Data presented at WFH 2018 - Filed in US (priority review) and EU in Q2 2018 - Data published in <i>NEJM</i> 2018; 379: 811-822	- Pharmacokinetic run-in data at ASH 2017 - Positive interim analysis outcome reported Q4 2017 - Data presented at WFH 2018 - Interim data filed in US and EU in Q2 2018 - Data published in <i>Lancet Haematology</i> 2019 Jun;6(6):e295-e305
	Approved in US Q4 2018 and EU Q1 2019	
CT Identifier	NCT02847637	NCT03020160

In collaboration with Chugai

ASH=American Society of Hematology; WFH=World Federation of Hemophilia; NEJM=New England Journal of Medicine

Hemlibra (emicizumab, RG6013)

Factor VIII mimetic for treatment of hemophilia A

Indication	Hemophilia A patients with and without inhibitors to Factor VIII	Hemophilia A mild to moderate patients without inhibitors to Factor VIII
Phase/study	Phase III HAVEN 5	Phase III HAVEN 6
# of patients	N=85	N=70
Design	Patients with Hemophilia regardless of FVIII inhibitor status on prophylactic or episodic treatment prior to study entry: ARM A: Hemlibra prophylaxis qw ARM B: Hemlibra prophylaxis q4w ARM C: No prophylaxis (control arm)	Patients with mild or moderate Hemophilia A without FVIII inhibitors Hemlibra qw (1.5mg/kg), q2w (3.0mg/kg) or q4w (6.0mg/kg) (patients preference)
Primary endpoint	Number of bleeds over 24 weeks	Safety and efficacy
Status	- FPI Q2 2018 - Recruitment completed Q1 2019 - Filed in China Q2 2020 - Approved in China Q2 2021	- FPI Q1 2020, recruitment completed Q1 2021 - Interim data presented at ASH 2021 and primary data presented at ISTH 2022 - Filed in EU Q4 2021 - Data presented at ASH 2022 - Approved in EU for moderate Hemophilia A Q1 2023
CT Identifier	NCT03315455	NCT04158648

Alecensa (alectinib, RG7853)

New CNS-active inhibitor of anaplastic lymphoma kinase

Indication	Treatment-naïve ALK+ advanced NSCLC	Adjuvant ALK+ NSCLC
Phase/study	Phase III ALEX	Phase III ALINA
# of patients	N=286	N=257
Design	ARM A: Alecensa 600mg BID ARM B: Crizotinib 250mg BID	ARM A: Alecensa 600mg BID ARM B: Platinum-based chemotherapy
Primary endpoint	Progression-free survival	Disease-free survival
Status	- Data presented at ASCO 2017, 2018, ESMO 2017, 2018 and 2019 (final PFS and updated OS) - Data published in <i>NEJM</i> 2017; 377:829-838 - Approved in US Q4 2017 (priority review) and in EU Q4 2017	- FPI Q3 2018 - Recruitment completed Q4 2021
CT Identifier	NCT02075840	NCT03456076

Kadcyla (trastuzumab emtansine, RG3502)

First ADC for HER2-positive breast cancer

Indication	HER2-positive early breast cancer (BC) high-risk patients	HER2-positive early breast cancer (BC) high-risk patients
Phase/study	Phase III KATHERINE	Phase III ASTEFANIA
# of patients	N=1,484	N=1,700
Design	- ARM A: Kadcyla 3.6mg/kg q3w - ARM B: Herceptin	- ARM A: Kadcyla plus Tecentriq - ARM B: Kadcyla plus placebo
Primary endpoint	Invasive disease-free survival	Invasive disease-free survival
Status	- Stopped at pre-planned interim data analysis for efficacy Q4 2018 - Data presented at SABCS 2018 - BTD granted by FDA in Q1 2019 - Filed in US (under RTOR) and EU Q1 2019 - Approved in US Q2 2019 and in EU Q4 2019 - Data published in <i>NEJM</i> 2019; 380:617-628	FPI Q2 2021
CT Identifier	NCT01772472	NCT04873362

Phesgo (pertuzumab/trastuzumab, RG6264)

FDC of Perjeta and Herceptin for subcutaneous administration

Indication	HER2-positive early breast cancer (BC)		HER2-positive breast cancer (BC)
Phase/study	Phase III FeDeriCa	Phase II PHranceSCa	Pivotal Phase I ¹
# of patients	N=500	N=160	N=144
Design	Phesgo in combination with chemotherapy in neoadjuvant/adjuvant setting ARM A: Perjeta IV plus Herceptin IV plus chemotherapy ARM B: Phesgo plus chemotherapy	ARM A: Perjeta and Herceptin IV followed by Phesgo ARM B: Phesgo followed by IV	ARM A: Phesgo administered using a handheld syringe with hypodermic needle (SC) ARM B: Phesgo administered using the on-body delivery system (OBI)
Primary endpoint	Trough Serum Concentration (C_{trough}) of Perjeta during cycle 7	Percentage of patients who preferred Phesgo	AUC₀₋₆₂*, C_{max}**
Status	- Primary endpoint met Q3 2019 - Data presented at SABCS 2019 - Data published in Lancet Oncology 2021 Jan;22(1):85-97	- Final analysis completed, 85% patients preferred Phesgo - Data presented at ESMO 2020 - Data published in Eur J Cancer 2021 Jul;152:223-232	FPI Q2 2022
	Filed in US Q4 2019 & in EU Q1 2020; Approved in US Q2 2020 and EU Q4 2020		
CT Identifier	NCT03493854	NCT03674112	NCT05275010

SC with Halozyme's rHuPH20/ Halozyme's human hyaluronidase; ¹In collaboration with West Pharmaceuticals; *AUC₀₋₆₂=comparability of area under the time-concentration curve from the start of dosing to 63 days; **C_{max}=maximum serum concentration for pertuzumab and trastuzumab within Phesgo; FDC=Fixed-dose combination; Phesgo=FDC of Perjeta and Herceptin for SC administration; HER2=Human Epidermal growth factor Receptor 2, IV=intravenous; SC=Subcutaneous; SABCS=San Antonio Breast Cancer Symposium; Eur J Cancer=European Journal of Cancer; ESMO=European Society for Medical Oncology

Tecentriq (atezolizumab, RG7446)

Anti-PD-L1 cancer immunotherapy – lung cancer

Indication	Adjuvant NSCLC	Perioperative NSCLC
Phase/study	Phase III IMpower010	Phase III IMpower030
# of patients	N=1,280	N=450
Design	Following adjuvant cisplatin-based chemotherapy ▪ ARM A: Tecentriq ▪ ARM B: Best supportive care	▪ ARM A: Tecentriq plus platinum-based chemotherapy ▪ ARM B: Platinum-based chemotherapy
Primary endpoint	▪ Disease-free survival	▪ Event-free survival
Status	▪ Recruitment completed Q3 2018 ▪ Study met primary endpoint Q1 2021 ▪ Data presented at ASCO, WCLC and ESMO 2021 ▪ Filed in US (priority review) and EU Q2 2021 ▪ Approved in US Q4 2021 and EU Q2 2022	▪ FPI Q2 2018 ▪ Recruitment completed Q3 2021
CT Identifier	NCT02486718	NCT03456063

NSCLC=non-small cell lung cancer; PD-L1=Programmed cell death-ligand 1; ASCO=American Society of Clinical Oncology; ESMO=European Society for Medical Oncology; WCLC=World Conference on Lung Cancer

Tecentriq (atezolizumab, RG7446)

Anti-PD-L1 cancer immunotherapy – lung cancer

Indication	1L maintenance extensive-stage SCLC	Stage IV NSCLC
Phase/study	Phase III IMforte ¹	Phase Ib/III IMscin001 ²
# of patients	N=450	N=371
Design	ARM A: Platinum-etoposide + Tecentriq followed by maintenance Tecentriq plus lurbinectedin ARM B: Platinum-etoposide + Tecentriq followed by maintenance Tecentriq	Phase Ib - Dose finding, Tecentriq SC followed by Tecentriq IV Phase III 2L NSCLC non inferiority of Tecentriq SC vs Tecentriq IV
Primary endpoint	Progression-free survival and overall survival	Observed concentration of Tecentriq in serum at cycle 1
Status	FPI Q4 2021	- FPI Phase Ib Q4 2018 and FPI Phase III Q4 2020 - Recruitment completed Q1 2022 - Study met its primary end point Q3 2022 - Data presented at ESMO-IO 2022 - Filed in US and EU Q4 2022
CT Identifier	NCT05091567	NCT03735121

¹In collaboration with Jazz Pharma, ²SC with Halozyme's rHuPH20/ Halozyme's human hyaluronidase

NSCLC=non-small cell lung cancer; PD-L1=Programmed cell death-ligand 1; SCLC=small cell lung cancer, SC=Subcutaneous, IV=Intravenous; ESMO-IO=European Society for Medical Oncology-Immuno-Oncology

Tecentriq (atezolizumab, RG7446)

Anti-PD-L1 cancer immunotherapy – SCCHN

Indication	Adjuvant squamous cell carcinoma of the head and neck (SCCHN)
Phase/study	Phase III IMvoke010
# of patients	N=406
Design	▪ ARM A: Tecentriq 1200mg q3w ▪ ARM B: Placebo
Primary endpoint	▪ Event-free survival and overall survival
Status	▪ FPI Q1 2018 ▪ Recruitment completed Q1 2020
CT Identifier	NCT03452137

SCCHN=squamous cell carcinoma of the head and neck

Tecentriq (atezolizumab, RG7446)

Anti-PD-L1 cancer immunotherapy – urothelial carcinoma

Indication	High-risk non-muscle-invasive bladder cancer (NMIBC)	ctDNA+, high-risk muscle-invasive bladder cancer (MIBC)
Phase/study	Phase III ALBAN	Phase III IMvigor011
# of patients	N=516	N=495
Design	- ARM A: BCG induction and maintenance - ARM B: Tecentriq plus BCG induction and maintenance	- ARM A: Tecentriq monotherapy - ARM B: Placebo
Primary endpoint	Recurrence-free survival	Recurrence-free survival
Status	FPI Q4 2018	FPI Q2 2021
CT Identifier	NCT03799835	NCT04660344

BCG=Bacille Calmette-Guérin; PD-L1=Programmed cell death-ligand 1

Tecentriq (atezolizumab, RG7446)

Anti-PD-L1 cancer immunotherapy – hepatocellular carcinoma

Indication	Adjuvant hepatocellular carcinoma (HCC)
Phase/study	Phase III IMbrave050
# of patients	N=668
Design	▪ ARM A: Tecentriq plus Avastin ▪ ARM B: Active surveillance
Primary endpoint	▪ Recurrence-free survival
Status	▪ FPI Q4 2019 ▪ Recruitment completed Q4 2021 ▪ Study met its primary endpoint Q1 2023 ▪ Data presented at AACR 2023 and ASCO 2023 (PROs)
CT Identifier	NCT04102098

Tecentriq (atezolizumab, RG7446)

Anti-PD-L1 cancer immunotherapy – breast cancer

Indication	Previously untreated metastatic triple negative breast cancer (TNBC)	
Phase/study	Phase III IMpassion130	Phase III IMpassion132
# of patients	N=902	N=572
Design	▪ ARM A: Tecentriq plus nab-paclitaxel ▪ ARM B: Placebo plus nab-paclitaxel	▪ ARM A: Tecentriq plus capecitabine or carbo/gem ▪ ARM B: Placebo plus capecitabine or carbo/gem
Primary endpoint	▪ Progression-free survival and overall survival (co-primary endpoint)	▪ Overall survival
Status	▪ Study met co-primary endpoint of PFS in both PD-L1+ and ITT populations Q3 2018 ▪ Primary PFS and interim OS data presented at ESMO 2018 and ASCO 2019 ▪ Data published in <i>NEJM</i> 2018; 379:2108-2121 ▪ US accelerated approval Q1 2019 – US indication voluntarily withdrawn Q3 2021 ▪ Approved in EU Q3 2019 ▪ Final OS presented at ESMO Asia 2020	▪ FPI Q1 2018
CT Identifier	NCT02425891	NCT03371017

Tecentriq (atezolizumab, RG7446)

Anti-PD-L1 cancer immunotherapy – breast cancer

Indication	Neoadjuvant triple negative breast cancer (TNBC)
Phase/study	Phase III IMpassion031
# of patients	N=333
Design	▪ ARM A: Tecentriq plus nab-paclitaxel ▪ ARM B: Placebo plus nab-paclitaxel
Primary endpoint	▪ Percentage of participants with pathologic complete response
Status	▪ Study met primary endpoint Q2 2020 ▪ Data presented at ESMO 2020 ▪ Data published in Lancet 2020;396 (10257):1090-1100 ▪ Filed in EU Q4 2020 - application withdrawn Q3 2021
CT Identifier	NCT03197935

Venclexta (venetoclax, RG7601)

Novel small molecule Bcl-2 selective inhibitor – chronic lymphocytic leukemia

Indication	Untreated chronic lymphocytic leukemia (CLL) patients with coexisting medical conditions	Untreated fit chronic lymphocytic leukemia (CLL) patients
Phase/study	Phase III CLL14	Phase III CristaLLO
# of patients	N=445	N=165
Design	ARM A: Venclexta plus Gazyva ARM B: Chlorambucil plus Gazyva	ARM A: Venclexta plus Gazyva ARM B: Fludarabine plus cyclophosphamide plus Rituxan or bendamustine plus Rituxan
Primary endpoint	Progression-free survival	MRD negativity rate in peripheral blood at 15 months
Status	- Study met primary endpoint Q4 2018 - BTD granted by FDA Q1 2019 - Filed in US (under RTOR) Q1 2019 and EU Q2 2019 - Data presented at ASCO 2019, ASH 2019, 2020 and EHA 2021, 2022; 6-year data presented at EHA and ICML 2023 - Data published in <i>NEJM</i> 2019; 380:2225-2236 - Approved US Q2 2019 and EU Q1 2020	- FPI Q2 2020 - Recruitment completed Q1 2023
CT Identifier	NCT02242942	NCT04285567

Venclexta (venetoclax, RG7601)

Novel small molecule Bcl-2 selective inhibitor – multiple myeloma

Indication	Relapsed or refractory multiple myeloma (MM)	
Phase/study	Phase I	Phase III CANOVA
# of patients	N=117	N=244
Design	▪ Dose escalation cohort: Venclexta dose escalation ▪ Safety expansion cohort (t11;14): Venclexta expansion ▪ Combination cohort: Venclexta plus dexamethasone	▪ ARM A: Venclexta plus dexamethazone ▪ ARM B: Pomalidomide plus dexamethasone in t(11;14) positive r/r MM
Primary endpoint	▪ Safety and maximum tolerated dose	▪ Progression-free survival
Status	▪ Data presented at ASCO 2015, 2016 and ASH 2016 ▪ Data published in Blood 2017; 130(22):2401-2409 and Am J Hematol 2021 Apr 1;96(4):418-427	▪ FPI Q4 2018 ▪ Recruitment completed Q3 2022
CT Identifier	NCT01794520	NCT03539744

Venclexta (venetoclax, RG7601)

Novel small molecule Bcl-2 selective inhibitor – myelodysplastic syndromes

Indication	Relapsed or refractory myelodysplastic syndromes (MDS)	Treatment-naïve myelodysplastic syndromes (MDS)	Newly diagnosed higher-risk myelodysplastic syndrome (MDS)
Phase/study	Phase Ib	Phase Ib	Phase III VERONA
# of patients	N=70	N=129	N=500
Design	Cohort 1: ARM A: Venclexta 400 mg ARM B: Venclexta 800 mg Cohort 2: - Venclexta plus azacitidine Study expansion: - Venclexta or Venclexta plus azacitidine	Dose escalation cohort: Venclexta plus azacitidine dose escalation Safety expansion cohort	ARM A: Venclexta plus azacitidine ARM B: Placebo plus azacitidine
Primary endpoint	Safety, efficacy, Pharmacokinetics and Pharmacodynamics	Safety, Pharmacokinetics, RPTD	Overall survival
Status	- FPI Q1 2017 - Recruitment completed Q1 2022 - Data published in <i>Am J Hematol</i> 2023 Feb;98(2):272-281	- FPI Q1 2017 - Data presented at ASH 2019, 2020 and ASCO 2021 - BTD granted by FDA July 2021 - Recruitment completed Q1 2022	- FPI Q4 2020 - Recruitment completed Q3 2022
CT Identifier	NCT02966782	NCT02942290	NCT04401748

Polivy (polatuzumab vedotin, RG7596)

ADC targeting CD79b to treat B cell malignancies

Indication	1L DLBCL
Phase/study	Phase III POLARIX
# of patients	N=879
Design	- ARM A: Polivy plus R-CHP - ARM B: R-CHOP
Primary endpoint	Progression-free survival
Status	- Data presented at ASH 2021 and 2022 - Filed in EU, Japan and China Q4 2021 and in the US Q3 2022 - Published in <i>NEJM</i> 2022 Jan 27;386(4):351-363 - Approved in EU Q2 2022, Japan Q3 2022, China Q1 2023 and US April 2023
CT Identifier	NCT03274492

Gavreto (pralsetinib, RG6396)

Highly selective RET inhibitor

Indication	RET+ NSCLC, thyroid cancer and other advanced solid tumors	1L RET fusion-positive, metastatic NSCLC
Phase/study	Phase I/II ARROW	Phase III AcceleRET Lung
# of patients	N=647	N=250
Design	▪ Part I: Gavreto 30-600mg dose escalation ▪ Part II: Gavreto 400mg dose expansion	▪ ARM A: Gavreto 400mg ▪ ARM B: Platinum-based chemotherapy +/- pembrolizumab
Primary endpoint	▪ Safety and efficacy	▪ Progression-free survival
Status	▪ Filed in US and EU for RET fusion-positive NSCLC and US for RET-mutant MTC and RET fusion-positive thyroid cancer ▪ Approved in US Q3 2020 in RET fusion-positive NSCLC, in Q4 2020 in RET-mutant MTC and RET fusion-positive thyroid cancer ▪ Updated data presented at ASCO 2021 and 2022 ▪ Data published in Lancet Oncol 2021 Jul;22(7):959-969 and Lancet Diabetes & Endocrinology Aug 2021;9(8):491-501 ▪ Approved in EU for RET fusion-positive NSCLC Q4 2021 ▪ Filing withdrawn in EU Q4 2022 for RET-mutant MTC and RET fusion-positive thyroid cancer ▪ US Approval withdrawn Q2 2023 for RET-mutant medullary thyroid cancer	▪ Study initiated in Q1 2020
CT Identifier	NCT03037385	NCT04222972

Lunsumio (mosunetuzumab, CD20 x CD3, RG7828)

Bispecific anti-CD20/CD3 antibody engaging T and B cells simultaneously

Indication	3L+ FL, 3L+ DLBCL & other relapsed or refractory NHL	1L DLBCL	Relapsed or refractory DLBCL
Phase/study	Phase I/II	Phase Ib/II	Phase Ib/II
# of patients	N=746	N=160	N=262
Design	- Dose escalation of Lunsumio monotherapy and in combination with Tecentriq - Expansion cohorts for r/r FL, r/r DLBCL and SC in r/r NHL	- Lunsumio plus CHOP - Lunsumio plus CHP plus Polivy - Lunsumio plus CHP-Polivy vs Rituximab plus CHP-Polivy	Lunsumio plus Polivy, randomised cohorts ARM A: Lunsumio SC plus Polivy ARM B: Rituximab plus Polivy
Primary endpoint	Safety, tolerability, dose/schedule, PK and response rates	Safety/tolerability and response	Safety/tolerability and response
Status	- Data in r/r NHL presented at ASH 2018, 2019, and in r/r FL at ASH 2020, 2021 and 2022 - BTD granted by FDA Q2 2020 - Filed in EU and rolling submission in US Q4 2021; Filed in US (priority review) Q2 2022 - Approved in EU Q2 2022 and US Q4 2022 - DLBCL data published in <i>J. Clin. Oncol.</i> 40(5)481-491 and <i>Blood Advances</i> 2023 Apr 17: doi:10.1182/bloodadvances.2022009260 - FL data published in the <i>Lancet Oncology</i> 2022 Aug;23(8):1055-1065	- FPI Q1 2019 - Recruitment completed Q2 2021 - Data for Lunsumio plus CHOP presented at ASH 2020	- FPI Q3 2018 - Recruitment completed Q1 2023 - Initial data presented at ASCO 2021 and ASH 2021, 2022
CT Identifier	NCT02500407	NCT03677141	NCT03671018

FL=follicular lymphoma; DLBCL=diffuse large B cell lymphoma; r/r=relapsed/refractory; NHL=non-Hodgkin's lymphoma; R=Rituximab; SC=subcutaneous; CHOP=cyclophosphamide, doxorubicin, vincristine, and prednisone; CHP=cyclophosphamide, doxorubicin, and prednisone; PK=Pharmacokinetics; BTD=Breakthrough Therapy Designation; ASH=American Society of Hematology; ASCO=American Society of Clinical Oncology

Lunsumio (mosunetuzumab, CD20 x CD3, RG7828)

Bispecific anti-CD20/CD3 antibody engaging T and B cells simultaneously

Indication	1L DLBCL & 2L DLBCL following 1L induction	Relapsed or refractory 2L+ FL
Phase/study	Phase I	Phase Ib
# of patients	N=188	N=27
Design	▪ Cohort A: Lunsumio monotherapy (after a response to prior systemic chemotherapy) ▪ Cohort B: Lunsumio monotherapy (1L treatment in elderly/frail) ▪ Cohort C: Lunsumio SC plus Polivy in 1L elderly/unfit	▪ Lunsumio plus lenalidomide safety run-in for phase III ▪ Lunsumio SC plus lenalidomide
Primary endpoint	▪ Safety/tolerability and response	▪ Safety/tolerability and response
Status	▪ FPI Q2 2019 – Cohort B ▪ FPI Q3 2019 – Cohort A ▪ FPI Q1 2021 – Cohort C ▪ Recruitment completed Q1 2023 ▪ Initial data presented at ASH 2020 (Cohort B) and ASH 2022	▪ FPI Q3 2020 ▪ Initial data presented at ASH 2021 and 2022 ▪ Recruitment completed Q2 2023
CT Identifier	NCT03677154	NCT04246086

Lunsumio (mosunetuzumab, CD20 x CD3, RG7828)

Bispecific anti-CD20/CD3 antibody engaging T and B cells simultaneously

Indication	2L+ FL	Relapsed or refractory CLL
Phase/study	Phase III CELESTIMO	Phase Ib/II
# of patients	N=400	N=56
Design	▪ ARM A: Lunsumio plus lenalidomide ▪ ARM B: Rituxan plus lenalidomide	▪ Lunsumio monotherapy (3L+ CLL)
Primary endpoint	▪ Progression-free survival	▪ Safety, dose-limiting toxicity and RPTD
Status	▪ FPI Q4 2021	▪ FPI Q1 2022
CT Identifier	NCT04712097	NCT05091424

FL=follicular lymphoma; r/r=relapsed/refractory; RPTD=Recommended Phase II Dose; CLL=Chronic lymphocytic leukemia

Lunsumio (mosunetuzumab, CD20 x CD3, RG7828)

Bispecific anti-CD20/CD3 antibody engaging T and B cells simultaneously

Indication	2L+ SCT ineligible DLBCL
Phase/study	Phase III SUNMO
# of patients	N=222
Design	- ARM A: Lunsumio plus Polivy - ARM B: R + GemOx
Primary endpoint	Progression-free survival
Status	FPI Q2 2022
CT Identifier	NCT05171647

DLBCL=diffuse large B cell lymphoma; SCT=stem cell transplant; R=Rituxan/MabThera; GemOx=Gemcitabin und Oxaliplatin

Columvi (glofitamab, CD20-TCB, RG6026)

Bispecific anti-CD20/CD3 antibody engaging T and B cells simultaneously

Indication	Relapsed or refractory Non-Hodgkin's lymphoma (NHL)		
Phase/study	Phase I	Phase Ib	Phase I
# of patients	N=700	N=140	N=18-36
Design	Cohort 1: Single-agent dose escalation study - Initial dose escalation - Expansion cohort in r/r DLBCL - Expansion cohort in r/r FL - All patients will receive pretreatment with a single dose of Gazyva (1000mg) Cohort 2: Columvi plus Gazyva (i.e. continuous treatment with Gazyva)	Dose escalation and expansion ARM A: Columvi plus Tecentriq ARM B: Columvi plus Polivy	Columvi SC Part 1 dose escalation
Primary endpoint	Efficacy, safety, tolerability and PK	Safety	Safety
Status	- Data presented at ASH 2018, 2020, 2021, 2022, ICML 2019, 2021, EHA 2020, 2021, 2022 and ASCO 2021, 2022 and 2023 - Data published in <i>J Clin Oncology</i> 2021; 39:18:1959-1970 and <i>NEJM</i> 2022; 387:2220-2231 - Filed in EU Q2 2022 and US Q4 2022 - Approved in Canada Q1 2023, US Q2 2023 and EU July 2023	ARM A: FPI Q2 2018 ARM B: FPI Q4 2020 - Recruitment completed Q2 2022 - Data presented at ASH 2019, 2021	FPI Q3 2021
CT Identifier	NCT03075696	NCT03533283	ISRCTN17975931

Columvi (glofitamab, CD20-TCB, RG6026)

Bispecific anti-CD20/CD3 antibody engaging T and B cells simultaneously

Indication	Non-Hodgkin's lymphoma (NHL)	2L+ SCT-ineligible DLBCL	1L ctDNA high risk DLBCL
Phase/study	Phase Ib	Phase III STARGLO	Phase II
# of patients	Part I: 15-60 Part II: ~66-104	N=270	N=40
Design	Part I: Dose-finding for the combination of Columvi plus G/R-CHOP in r/r indolent NHL Part II: Dose expansion Columvi plus G/R-CHOP or R-CHOP in 1L DLBCL Part III: Columvi plus R-CHP plus Polivy	ARM A: Columvi plus gemcitabine and oxaliplatin, followed by up to 4 cycles of Columvi monotherapy ARM B: Rituxan in combination with gemcitabine and oxaliplatin A single dose of Gazyva will be administered 7 days prior to the first dose of Columvi	Columvi plus R-CHOP (Columvi is introduced as a consolidation to R-CHOP at cycle 3-8 in patients ctDNA+ at cycle 2)
Primary endpoint	Safety	Overall survival	EOT PET-CR
Status	- Part I: FPI Q1 2018 - Part II: FPI Q1 2021 - Recruitment completed Q1 2023 - Data presented at ASH 2021, 2022 and ASCO 2023	- FPI Q1 2021 - Recruitment completed Q1 2023	FPI Q1 2022
CT Identifier	NCT03467373	NCT04408638	NCT04980222

Columvi (glofitamab, CD20-TCB, RG6026)

Bispecific anti-CD20/CD3 antibody engaging T and B cells simultaneously

Indication	2L+ SCT-eligible DLBCL	2L+ SCT-ineligible DLBCL
Phase/study	Phase Ib	Phase Ib
# of patients	N=40	N=112
Design	Columvi plus R-ICE (single-arm study)	- Columvi IV plus CELMoD (CC-220 and CC-99282) - Lunsumio SC plus CELMoD (CC-220 and CC-99282)
Primary endpoint	Objective response rate within 3 cycles	Safety, DLT, RPTD
Status	FPI Q4 2022	FPI Q4 2022
CT Identifier	NCT05364424	NCT05169515

DLBCL=diffuse large B cell lymphoma; DLT=Dose-limiting toxicity, RPTD=Recommended Phase II Dose; R-ICE= Rituxan plus ifosfamide, carboplatin, and etoposide; IV=Intravenous; SC=Subcutaneous

Ocrevus (ocrelizumab, RG1594)

Humanized monoclonal antibody selectively targeting CD20+ B cells

Indication	Relapsing multiple sclerosis (RMS)		Primary progressive multiple sclerosis (PPMS)
Phase/study	Phase III OPERA I	Phase III OPERA II	Phase III ORATORIO
# of patients	N=821	N=835	N=732
Design	96-week treatment period: ARM A: Ocrevus 2x300mg IV followed by 600mg IV q24w ARM B: Interferon β-1a (Rebif)	96-week treatment period: ARM A: Ocrevus 2x300mg IV followed by 600mg IV q24w ARM B: Interferon β-1a (Rebif)	120-week treatment period: ARM A: Ocrevus 2x300mg IV q24w ARM B: Placebo
Primary endpoint	Annualized relapse rate at 96 weeks versus Rebif	Annualized relapse rate at 96 weeks versus Rebif	Sustained disability progression versus placebo by EDSS
Status	- Primary endpoint met Q2 2015, OLE ongoing - Data presented atECTRIMS 2015, AAN andECTRIMS 2017, AAN and EAN 2018 - Data published in <i>NEJM</i> 2017; 376:221-234 - Data published on COVID-19 in <i>Mult Scler Relat Disord</i> on Ocrevus treated people with MS, doi.org/10.1016/j.msard.2020.102725		- Primary endpoint met Q3 2015 - Data presented atECTRIMS 2015, AAN andECTRIMS 2017, AAN and EAN 2018 - Data published in <i>NEJM</i> 2017; 376:209-220
	Approved in US Q1 2017 and EU Q1 2018		
CT Identifier	NCT01247324	NCT01412333	NCT01194570

Ocrevus (ocrelizumab, RG1594)

Humanized monoclonal antibody selectively targeting CD20+ B cells

Indication	Relapsing and primary progressive multiple sclerosis (RMS & PPMS)	Primary progressive multiple sclerosis (PPMS)
Phase/study	Phase IIIb ENSEMBLE PLUS	Phase IIIb ORATORIO-HAND
# of patients	N=1,225	N ~ 1,000
Design	- Substudy of ongoing phase IIIb, open-label, single-arm ENSEMBLE study - Shorter two-hour infusion time	120-week treatment period: ARM A: Ocrevus 600mg IV q24w ARM B: Placebo
Primary endpoint	Safety, measured by the proportion of patients with IRRs following the first randomised 600 mg infusion	Time to upper limb disability progression confirmed for at least 12 weeks
Status	- Filed in US and EU Q1 2020 - Approved in EU Q2 2020 and US Q4 2020 - Data published <i>Neurol</i>, <i>Neuroimmunol</i> and <i>Neuroinflamm</i> Sept 2020; 7(5), e807	FPI Q3 2019
CT Identifier	NCT03085810	NCT04035005

IV=intravenous; IRR=Infusion Related Reaction

Ocrevus (ocrelizumab, RG1594)

Humanized monoclonal antibody selectively targeting CD20+ B cells

Indication	Primary progressive multiple sclerosis (PPMS)	Relapsing multiple sclerosis (RMS)	PPMS & RMS
Phase/study	Phase IIIb GAVOTTE	Phase IIIb MUSSETTE	Phase III Ocarina II ¹
# of patients	N ~ 699	N ~ 786	N ~ 232
Design	120-week treatment period: ▪ ARM A: Ocrevus 600mg IV q24w ▪ ARM B: Ocrevus 1200mg if BW <75kg or 1800mg if BW ≥75kg q24w	120-week treatment period: ▪ ARM A: Ocrevus 600mg IV q24w ▪ ARM B: Ocrevus 1200mg if BW <75kg or 1800mg if BW ≥75kg q24w	▪ ARM A: Ocrevus IV ▪ ARM B: Ocrevus SC
Primary endpoint	▪ Superiority of Ocrevus higher dose versus approved dose on cCDP	▪ Superiority of Ocrevus higher dose versus approved dose on cCDP	▪ Serum Ocrevus area under the concentration-time curve (AUCW1-12) at week 12
Status	▪ FPI Q4 2020 ▪ Recruitment completed Q2 2023	▪ FPI Q4 2020 ▪ Recruitment completed Q4 2021	▪ FPI Q2 2022 ▪ Recruitment completed Q4 2022 ▪ Study met it's primary endpoint July 2023
CT Identifier	NCT04548999	NCT04544436	NCT05232825

¹SC with Halozyme's rHuPH20/ Halozyme's human hyaluronidase
cCDP=composite confirmed disability progression; IV=intravenous; SC=Subcutaneous

Evrysdi (risdiplam, RG7916)

Oral SMN2 splicing modifier

Indication	Spinal muscular atrophy (SMA)		
Phase/study	Phase II/III FIREFISH	Phase II/III SUNFISH	Phase II JEWELFISH
# of patients	N=21 (Part 1), 41 (Part 2)	N=51 (Part 1), 180 (Part 2)	N=174
Design	Infants with type 1 SMA ▪ Part I (dose-finding): ≥4 weeks ▪ Part II (confirmatory): 24 months	Adult & pediatric patients with type 2 or 3 SMA: ▪ Part I (dose-finding): At least 12 weeks ▪ Part II (confirmatory): 24 months	▪ Adult and pediatric patients with previously treated SMA type 1, 2 and 3
Primary endpoint	▪ Safety, tolerability, PK/PD and efficacy	▪ Safety, tolerability, PK/PD and efficacy	▪ Safety, tolerability, PK/PD
Status	▪ Part I 12-month data presented at AAN, CureSMA and EAN 2019; 16-month data presented at WMS 2019 ▪ Part II 1-year data presented at AAN 2020, Part I 2-year data at WMS 2020 ▪ Part I data published in <i>NEJM</i> 2021;384:915-923 ▪ Part II 2-year data presented at AAN 2021 ▪ Part II 1-year data published in <i>NEJM</i> 2021;385:427-435 ▪ 3-year data presented at EPNS 2022 and 4-year data presented at Cure SMA and EAN 2023	▪ Part I 12-month data presented at AAN, CureSMA and EAN 2019; 16-month data presented at WMS 2019 ▪ Part II 1-year data presented at SMA Europe 2020, 2-year data at MDA 2021, 3-year data at MDA 2022 and 4-year data at MDA and EAN 2023 ▪ Part II 1-year data published in <i>Lancet Neurology</i>, 2022; 21 (1) 42-52	▪ Data presented at WMS 2017, AAN 2018, WMS 2018, CureSMA 2019, WMS 2019, CureSMA 2020 and 2021 ▪ 2-year data presented at WMS 2022
CT Identifier	NCT02913482	NCT02908685	NCT03032172

Evrysdi (risdiplam, RG7916)

Oral SMN2 splicing modifier

Indication	Spinal muscular atrophy (SMA)
Phase/study	Phase II RAINBOWFISH
# of patients	N=25
Design	Infants aged from birth to 6 weeks who have been genetically diagnosed with SMA but are not yet presenting with symptoms
Primary endpoint	Proportion of participants with two copies of the SMN2 gene and baseline CMAP$\geq$1.5 millivolt who are sitting without support
Status	- FPI Q3 2019 - Recruitment completed Q1 2022 - Initial data presented at CureSMA , WMS 2021, MDA and WMS 2022 - Filed in US and EU Q4 2021 - Approved in US Q2 2022
CT Identifier	NCT03779334

In collaboration with PTC Therapeutics and SMA Foundation
 SMN=survival motor neuron; CMAP=compound muscle action potential; WMS=World Muscle Society; CureSMA=Annual SMA Conference; MDA=Muscular Dystrophy Association;

Enspryng (satralizumab, RG6168, SA237)

Anti-IL-6 receptor humanized monoclonal antibody

Indication	Neuromyelitis optica spectrum disorder (NMOSD)	
Phase/study	Phase III SAkuraStar	Phase III SAkuraSky
# of patients	N=95	N=83
Design	Enspryng monotherapy: ARM A: Enspryng 120mg SC monthly ARM B: Placebo SC monthly	Add-on therapy of Enspryng: ARM A: Enspryng 120mg SC monthly ARM B: Placebo SC monthly - Both arms on top of baseline therapies: azathioprine, mycophenolate mofetil or oral corticosteroids
Primary endpoint	Efficacy (time to first relapse), safety and PK/PD	Efficacy (time to first relapse), safety and PK/PD
Status	- Primary endpoint met Q4 2018 - Data presented at ECTRIMS 2019 - Published in Lancet Neurology 2020; 19(5): 402-412	- Primary endpoint met Q3 2018 - Data presented at ECTRIMS 2018 and AAN 2019 - Published in NEJM 2019; 381:2114-2124
CT Identifier	NCT02073279	NCT02028884

Trials managed by Chugai (Roche opted-in)
 BTD=Breakthrough therapy designation; PK/PD=Pharmacokinetics/Pharmacodynamics; SC=Subcutaneous; ECTRIMS=European Committee for Treatment and Research in Multiple Sclerosis; AAN=American Academy of Neurology; NEJM=New England Journal of Medicine

Enspryng (satralizumab, RG6168, SA237)

Anti-IL-6 receptor humanized monoclonal antibody

Indication	Generalised myasthenia gravis (MG)	Myelin oligodendrocyte glycoprotein antibody disease (MOG-AD)	Autoimmune encephalitis (AIE)
Phase/study	Phase III Luminesce	Phase III METEOROID	Phase III CIELO
# of patients	N=240	N=152	N=152
Design	ARM A: Enspryng plus standard of care ARM B: Placebo plus standard of care	ARM A: Enspryng at weeks 0, 2, 4 (loading doses) and maintenance doses q4w ARM B: Placebo	ARM A: Enspryng at weeks 0, 2, 4 (loading doses) and maintenance doses q4w ARM B: Placebo
Primary endpoint	Mean change from baseline in total MG-ADL score at week 24 in AChR+ population	Time from randomization to the first occurrence of a MOG-AD relapse	Efficacy (proportion of participants with mRS score improvement ≥ 1 from baseline and no use of rescue therapy at week 24) and safety
Status	- ODD granted in US Q1 2021 - FPI Q4 2021	- FPI Q3 2022 - ODD granted by FDA in Q4 2021	- FPI Q3 2022 - ODD granted for NMDAR AIE in US Q3 22
CT Identifier	NCT04963270	NCT05271409	NCT05503264

TNKase (RG3625, tenecteplase)

Small molecule tissue plasminogen activator

Indication	Stroke patients between 4.5 and 24 hours
Phase/study	Phase III TIMELESS
# of patients	N=456
Design	▪ ARM A: Tenecteplase (0.25 mg/kg, maximum 25 mg) single bolus injection ▪ ARM B: Placebo
Primary endpoint	▪ Ordinal modified Rankin scale (mRS) score after 90 days
Status	▪ FPI Q1 2019 ▪ Recruitment completed Q4 2022 ▪ Study did not meet it's primary endpoint Q2 2023
CT Identifier	NCT03785678

Gazyva (obinutuzumab, RG7159)

Immunology development program

Indication	Lupus nephritis		Membranous nephropathy
Phase/study	Phase II NOBILITY	Phase III REGENCY	Phase III MAJESTY
# of patients	N=126	N=252	N=140
Design	ARM A: Gazyva 1000mg IV plus MFF / mycophenolic acid ARM B: Placebo IV plus MFF/ mycophenolic acid	ARM A: Gazyva 1000mg IV (6 doses through Week 52) plus MFF ARM B: Gazyva 1000 mg IV (5 doses through Week 52) plus MFF ARM C: Placebo IV plus MFF	ARM A: Gazyva 1000mg IV on top of renin-angiotensin inhibitors ARM B: Tacrolimus treatment for 12 months
Primary endpoint	Percentage of participants who achieve complete renal response (CRR)	Percentage of participants who achieve complete renal response (CRR)	Percentage of patients who achieve complete remission at week 104
Status	- Primary endpoint met Q2 2019 - BTD granted by the FDA Q3 2019 - Data presented at ASN and ACR 2019 - Published in <i>Ann Rheum Dis</i> 2022 Jan;81(1):100-107	- FPI Q3 2020 - Recruitment completed Q1 2023	FPI Q2 2021
CT Identifier	NCT02550652	NCT04221477	NCT04629248

In collaboration with Biogen

BTD=Breakthrough therapy designation; IV=Intravenous; ASN=American Society of Nephrology; ACR=American College of Rheumatology; MFF=mycophenolate mofetil

Gazyva (obinutuzumab, RG7159)

Immunology development program

Indication	Systemic lupus erythematosus (SLE)	Childhood onset idiopathic nephrotic syndrome*
Phase/study	Phase III ALLEGORY	Phase III INShore
# of patients	N=200	N=80
Design	- ARM A: Gazyva 1000mg IV on Day 1 and Weeks 2, 24 and 26. - ARM B: Placebo IV	- ARM A: Gazyva plus oral steroids - ARM B: Mycophenolate mofetil (MMF) plus oral steroids
Primary endpoint	Percentage of participants who achieve Systemic Lupus Erythematosus Responder Index (SRI) at week 52	Percentage of participants with sustained complete remission at 1 year
Status	FPI Q4 2021	FPI Q1 2023
CT Identifier	NCT04963296	NCT05627557

In collaboration with Biogen
 *also known as pediatric nephrotic syndrome (PNS); IV=Intravenous

Lunsumio (mosunetuzumab, CD20 x CD3, RG7828)

Bispecific anti-CD20/CD3 antibody engaging T and B cells simultaneously

Indication	Systemic lupus erythematosus (SLE)
Phase/study	Phase I
# of patients	N=50
Design	▪ ARM A: Lunsumio SC on either Day 1 or on Days 1 and 8 ▪ ARM B: Fractionated (divided) dose of Lunsumio SC on Days 1 and 8
Primary endpoint	▪ Safety
Status	▪ FPI Q1 2022
CT Identifier	NCT05155345

Xolair (omalizumab, RG3648)

Humanized monoclonal antibody that selectively binds to IgE

Indication	Food allergy
Phase/study	Phase III OUtMATCH ¹
# of patients	N=225
Design	Xolair by SC injection either q2w or q4w for 16 to 20 weeks
Primary endpoint	Number of participants who successfully consume ≥600mg of peanut protein without dose-limiting symptoms
Status	FPI Q3 2019
CT Identifier	NCT03881696

In collaboration with Novartis; ¹ Sponsor of the study is the National Institute of Allergy and Infectious Diseases (NIAID)
IgE=Immunoglobulin E; SC=Subcutaneous

Susvimo (PDS, RG6321)

First eye implant to achieve sustained delivery of a biologic medicine

Indication	Wet age-related macular degeneration (wAMD)		
Phase/study	Phase III Archway	Phase II+III extension Portal	Phase IIIb Velodrome
# of patients	N=418	N=1,000	N=442
Design	- ARM A: PDS q24w - ARM B: Intravitreal ranibizumab q4w	Patients from LADDER or Archway receive refills of ranibizumab q24w (patients without the PDS will receive the PDS and subsequent refills)	- ARM A: PDS q36w - ARM B: PDS q24w
Primary endpoint	Change in BCVA from baseline at the average of week 36 and week 40	Safety and long term efficacy	Change in BCVA from baseline averaged over weeks 68 and 72
Status	- Study met primary endpoint Q2 2020 - Data presented at ASRS 2020, 44/48 week data at Angiogenesis 2021 and 2-year data at Angiogenesis 2022 - Filed in US (PRIME) and EU Q2 2021 - Approved in US Q4 2021	FPI Q3 2018	FPI Q2 2021
CT Identifier	NCT03677934	NCT03683251	NCT04657289

Susvimo (PDS, RG6321)

First eye implant to achieve sustained delivery of a biologic medicine

Indication	Diabetic macular edema (DME)	Diabetic retinopathy (DR) without center-involved diabetic macular edema (DME)
Phase/study	Phase III Pagoda	Phase III Pavilion
# of patients	N=545	N=160
Design	ARM A: PDS q24w ARM B: Intravitreal ranibizumab q4w	ARM A: Intravitreal ranibizumab (X2) followed by PDS implant (refill q36w) ARM B: Q4w comprehensive clinical monitoring until participants receive PDS (refill q36w)
Primary endpoint	Change in BCVA from baseline at the average of week 48 and week 52	Percentage of participants with a ≥ 2-step improvement from baseline on the ETDRS-DRSS at Week 52
Status	- FPI Q3 2019 - Recruitment completed Q2 2021 - Study met its primary endpoint Q4 2022 - Data presented at Angiogenesis 2023	- FPI Q3 2020 - Recruitment completed Q3 2021 - Study met its primary endpoint Q4 2022 - Data presented at Angiogenesis 2023
CT Identifier	NCT04108156	NCT04503551

Vabysmo (faricimab, RG7716)

Bispecific antibody to simultaneously bind Ang-2 and VEGF-A

Indication	Center-involving diabetic macular edema (CI-DME)	
Phase/study	Phase III YOSEMITE	Phase III RHINE
# of patients	N=940	N=951
Design	ARM A: Faricimab q8w ARM B: Faricimab PTI up to q16w ARM C: Aflibercept, q8w	ARM A: Faricimab q8w ARM B: Faricimab PTI up to q16w ARM C: Aflibercept, q8w
Primary endpoint	Change from baseline in BCVA at 1 year	Change from baseline in BCVA at 1 year
Status	- Study met primary endpoint Q4 2020 - Data presented at Angiogenesis 2021	- Study met primary endpoint Q4 2020 - Data presented at Angiogenesis 2021
CT Identifier	NCT03622580	NCT03622593

Ang-2=Angiopoietin-2; VEGF=Vascular endothelial growth factor; PTI=Personalized Treatment Interval; BCVA=best corrected visual acuity, ARVO=Association for Research in Vision and Ophthalmology

Vabysmo (faricimab, RG7716)

Bispecific antibody to simultaneously bind Ang-2 and VEGF-A

Indication	Wet age related macular degeneration (wAMD)	
Phase/study	Phase III TENAYA	Phase III LUCERNE
# of patients	N=671	N=658
Design	ARM A: Faricimab 6.0mg q16w flexible after 4 IDs ARM B: Aflibercept 2.0mg q8w after 3 IDs	ARM A: Faricimab 6.0mg q16w flexible after 4 IDs ARM B: Aflibercept 2.0mg q8w after 3 IDs
Primary endpoint	Change from baseline in BCVA week 40, 44 & 48	Change from baseline in BCVA week 40, 44 & 48
Status	- Study met primary endpoint Q1 2021 - Data presented at Angiogenesis 2021	- Study met primary endpoint Q1 2021 - Data presented at Angiogenesis 2021
CT Identifier	NCT03823287	NCT03823300

Vabysmo (faricimab, RG7716)

Bispecific antibody to simultaneously bind Ang-2 and VEGF-A

Indication	Macular edema (ME) secondary to branch retinal vein occlusion (RVO)	Macular edema (ME) secondary to central retinal vein occlusion (RVO)
Phase/study	Phase III BALATON	Phase III COMINO
# of patients	N=570	N=750
Design	▪ ARM A: Faricimab, q4w/PTI ▪ ARM B: Aflibercept, q4w	▪ ARM A: Faricimab, q4w/PTI ▪ ARM B: Aflibercept, q4w
Primary endpoint	▪ Change from baseline in BCVA at week 24	▪ Change from baseline in BCVA at week 24
Status	▪ FPI Q1 2021 ▪ Recruitment completed Q1 2022 ▪ Study met its primary endpoint Q4 2022 ▪ Data presented at Angiogenesis 2023 ▪ Filed in US Q2 2023	▪ FPI Q1 2021 ▪ Recruitment completed Q1 2022 ▪ Study met its primary endpoint Q4 2022 ▪ Data presented at Angiogenesis 2023 ▪ Filed in US Q2 2023
CT Identifier	NCT04740905	NCT04740931

Xofluza (baloxavir marboxil, RG6152, S-033188)

Small molecule, novel CAP-dependent endonuclease inhibitor

Indication	Influenza		
Phase/study	Phase III miniSTONE 1 (0-1 year old)	Phase III miniSTONE 2 (1- <12 years old)	Phase IIIb CENTERSTONE
# of patients	N=30	N=176	N=3,160
Design	Healthy pediatric patients from birth to <1 year with influenza-like symptoms receive Xofluza on Day 1	Healthy pediatric patients 1 to <12 years of age with influenza-like symptoms ARM A: Xofluza ARM B: Tamiflu	Reduction of direct transmission of influenza from otherwise healthy patients to household contacts ARM A: Xofluza ARM B: Placebo
Primary endpoint	Safety	Safety	Percentage of household contacts who are PCR-positive for influenza by day 5 post randomization of index patients
Status	FPI Q1 2019	- Primary endpoint met Q2 2019 - Data presented at OPTIONS X 2019 - Filed in US Q1 2020 and EU Q4 2021 - Data published in <i>Pediatric Infectious Disease</i> 2020 Aug;39(8):700-705 - Approved in the US (age 5 years and older) Q3 2022 , EU Jan 2023 and China (age 5 years and older) Q1 2023	FPI Q4 2019
CT Identifier	NCT03653364	NCT03629184	NCT03969212

Roche Group development pipeline

Marketed products development programmes

Roche Pharma global development programmes

Roche Pharma research and early development (pRED)

Genentech research and early development (gRED)

Spark

Tiragolumab (anti-TIGIT, RG6058, MTIG7192A)

Monoclonal antibody targeting the immune checkpoint inhibitor TIGIT

Indication	1L NSCLC PD-L1 TPS>50%	Stage III unresectable 1L NSCLC
Phase/study	Phase III SKYSCRAPER-01	Phase III SKYSCRAPER-03
# of patients	N=500-560	N=800
Design	ARM A: Tiragolumab plus Tecentriq ARM B: Placebo plus Tecentriq	ARM A: Tiragolumab plus Tecentriq for up to 12 months ARM B: Durvalumab for up to 12 months
Primary endpoint	Overall survival and progression-free survival	Progression-free survival
Status	- FPI Q1 2020 - Recruitment completed Q3 2021 - Study did not meet one of its primary endpoints, PFS, Q2 2022	- FPI Q3 2020 - Recruitment completed Q2 2023
CT Identifier	NCT04294810	NCT04513925

NSCLC=Non-small cell lung cancer; PD-L1=Programmed cell death-ligand 1; TPS=Tumor Proportion Score; PFS=Progression-free survival

Tiragolumab (anti-TIGIT, RG6058, MTIG7192A)

Monoclonal antibody targeting the immune checkpoint inhibitor TIGIT

Indication	Metastatic and/or recurrent PD-L1+ cervical cancer (CC)	Neoadjuvant and adjuvant NSCLC	1L non-squamous NSCLC
Phase/study	Phase II SKYSCRAPER-04	Phase II SKYSCRAPER-05	Phase III SKYSCRAPER-06
# of patients	N=172	N=82	N=540
Design	ARM A: Tiragolumab plus Tecentriq ARM B: Tecentriq	ARM A: (PD-L1 high) neoadjuvant tiragolumab plus Tecentriq followed by adjuvant tiragolumab plus Tecentriq or adjuvant chemotherapy ARM B: (PD-L1 all-comers) neoadjuvant tiragolumab plus Tecentriq plus chemo followed by adjuvant tiragolumab plus Tecentriq	ARM A: Tiragolumab plus Tecentriq plus pemetrexed plus chemotherapy followed by maintenance tiragolumab plus Tecentriq plus pemetrexed ARM B: Placebo plus pembrolizumab plus pemetrexed plus chemotherapy followed by maintenance placebo plus pembrolizumab plus pemetrexed
Primary endpoint	Objective response rate	Pathologic complete response, major pathological response and safety	Objective response rate, progression-free survival and overall survival
Status	FPI Q2 2020	FPI Q2 2021	FPI Q4 2020
CT Identifier	NCT04300647	NCT04832854	NCT04619797

Tiragolumab (anti-TIGIT, RG6058, MTIG7192A)

Monoclonal antibody targeting the immune checkpoint inhibitor TIGIT

Indication	Locally advanced esophageal cancer (EC)	1L esophageal cancer (EC)	1L recurrent/metastatic PD-L1 positive squamous cell head and neck carcinoma (SCCHN)
Phase/study	Phase III SKYSCRAPER-07	Phase III SKYSCRAPER-08	Phase II SKYSCRAPER-09
# of patients	N=750	N=500	N=120
Design	ARM A: Tiragolumab plus Tecentriq ARM B: Tecentriq plus placebo ARM C: Placebo plus placebo	ARM A: Tiragolumab plus Tecentriq plus cisplatin and paclitaxel ARM B: Placebo plus placebo plus cisplatin and paclitaxel	ARM A: Tiragolumab plus Tecentriq ARM B: Tecentriq plus placebo
Primary endpoint	- Progression-free survival (A vs C) - Overall survival (A vs C, hierarchical, B vs C hierarchical)	Overall survival and progression-free survival	Objective response rate
Status	FPI Q3 2020	- FPI Q4 2020 - Recruitment completed Q4 2021	- FPI Q1 2021 - Recruitment completed Q2 2022
CT Identifier	NCT04543617	NCT04540211	NCT04665843

Tiragolumab (anti-TIGIT, RG6058, MTIG7192A)

Monoclonal antibody targeting the immune checkpoint inhibitor TIGIT

Indication	Locally advanced, recurrent or metastatic solid tumors
Phase/study	Phase II SKYSCRAPER-11
# of patients	N=60
Design	Tiragolumab plus Tecentriq IV FDC
Primary endpoint	Safety
Status	FPI Q2 2023
CT Identifier	NCT05661578

NSCLC=Non-small cell lung cancer; FDC=Fixed-dose combination; IV=Intravenous

Tiragolumab (anti-TIGIT, RG6058, MTIG7192A)

Monoclonal antibody targeting the immune checkpoint inhibitor TIGIT

Indication	Solid tumors	NSCLC	Relapsed or refractory multiple myeloma (MM) or r/r B-cell NHL
Phase/study	Phase I	Phase II CITYSCAPE	Phase I
# of patients	N=540	N=135	N=52
Design	Phase Ia: Dose escalation and expansion of tiragolumab Phase Ib: Dose escalation and expansion of tiragolumab in combination with Tecentriq and/or other anti-cancer therapies	ARM A: Tecentriq plus tiragolumab ARM B: Tecentriq monotherapy	Phase Ia: Tiragolumab monotherapy Phase Ib: Tiragolumab plus daratumumab (r/r MM) or rituximab (r/r NHL)
Primary endpoint	Safety, tolerability, PK variability and preliminary efficacy	Overall response rate and progression-free survival	Safety, tolerability, PK/PD and preliminary efficacy
Status	Data presented at AACR 2020	- Data presented at ASCO 2020 and WCLC and ESMO IO 2021 - BTD granted by FDA Q4 2020 - Data published in <i>Lancet Oncol</i> 2022 Jun;23(6):781-792	FPI Q2 2019
CT Identifier	NCT02794571	NCT03563716	NCT04045028

Inavolisib (RG6114, GDC-0077)

A potent, orally available, and selective PI3K α inhibitor

Indication	PIK3CA-mutant HR-positive metastatic breast cancer (mBC)	post CDKi HR-positive breast cancer	PIK3CA mutant solid tumors and metastatic ER+ HER2-negative breast cancer
Phase/study	Phase III INAVO120	Phase III INAVO121	Phase I
# of patients	N=400	N=400	N=256
Design	ARM A: Inavolisib plus palbociclib plus fulvestrant ARM B: Placebo plus palbociclib plus fulvestrant	ARM A: Inavolisib plus fulvestrant ARM B: alpelisib plus fulvestrant	Monotherapy and in combination with standard of care (letrozole; letrozole plus palbociclib; fulvestrant) Stage 1: Dose escalation Stage 2: Dose expansion
Primary endpoint	Progression-free survival	Progression-free survival	Safety, tolerability and pharmacokinetics
Status	FPI Q1 2020	FPI Q2 2023	- FPI Q4 2016 - Preclinical/molecule discovery data presented at AACR 2017 - Data presented at SABCS 2019, 2020 and 2021
CT Identifier	NCT04191499	NCT05646862	NCT03006172

Giredestrant (SERD (3),RG6171, GDC-9545)

A selective estrogen receptor degrader or downregulator

Indication	ER+ HER2-negative metastatic breast cancer (mBC)	ER+ HER2-negative Stage I-III operable breast cancer (BC)	Neoadjuvant ER-positive breast cancer (BC)
Phase/study	Phase I	Phase I	Phase II coopERA Breast Cancer
# of patients	N=181	N=75	N=221
Design	- Dose escalation and expansion at RPTD - Giredestrant monotherapy and in combination with palbociclib and/or LHRH agonist	- Open-label, pre-operative administration - Dose escalation	ARM A: Giredestrant followed by giredestrant plus palbociclib ARM B: Anastrozole followed by anastrozole plus palbociclib
Primary endpoint	Safety	Safety, tolerability and PK/PD	Safety, tolerability and PK/PD
Status	- FPI Q4 2017 - Data presented at SABCS 2019, 2021 and ASCO 2020, 2021	- FPI Q3 2019 - Data presented at ASCO 2021	- FPI Q3 2020 - Data presented at ESMO and SABCS 2021; ASCO 2022 - Data (biomarker subgroup analysis) presented at ESMO 2022
CT Identifier	NCT03332797	NCT03916744	NCT04436744

Giredestrant (SERD (3),RG6171, GDC-9545)

A selective estrogen receptor degrader or downregulator

Indication	1L ER-positive metastatic breast cancer (mBC)	Adjuvant ER-positive breast cancer (BC)
Phase/study	Phase III persevERA Breast Cancer	Phase III lidERA Breast Cancer
# of patients	N=978	N=4,100
Design	- ARM A: Giredestrant plus palbociclib - ARM B: Letrozole plus palbociclib	- ARM A: Giredestrant monotherapy - ARM B: Tamoxifen or aromatase inhibitor
Primary endpoint	Progression-free survival	Invasive disease-free survival
Status	- FPI Q4 2020 - Recruitment completed Q1 2023	FPI Q3 2021
CT Identifier	NCT04546009	NCT04961996

ER=Estrogen receptor; HER2=Human Epidermal growth factor Receptor

Giredestrant (SERD (3),RG6171, GDC-9545)

A selective estrogen receptor degrader or downregulator

Indication	1L ER-positive/HER2-positive breast cancer (BC)	Grade 1 endometrial cancer
Phase/study	Phase III heredERA	Phase II endomERA
# of patients	N=812	N=45
Design	Induction Phesgo plus taxane followed by maintenance with either: ▪ ARM A: Giredestrant plus Phesgo ▪ ARM B: Phesgo	▪ Giredestrant once a day (QD) on days 1 to 28 of each 28-day cycle for 6 cycles
Primary endpoint	▪ Progression-free survival	▪ Percentage of participants who have regression by 6 months
Status	▪ FPI Q2 2022	▪ FPI Q2 2023
CT Identifier	NCT05296798	NCT05634499

ER=Estrogen receptor; HER2=Human Epidermal growth factor Receptor; Phesgo=FDC of Perjeta and Herceptin for SC administration

Divarasib (KRAS G12C inhibitor, RG6330, GDC-6036)

A potent, orally available, and selective inhibitor of the KRAS G12C mutant protein

Indication	Advanced or metastatic solid tumors with a KRAS G12C mutation	2L NSCLC	2L, 1L metastatic colorectal cancer (mCRC)
Phase/study	Phase I	Phase II/III B-FAST*	Phase Ib INTRINSIC
# of patients	N=438	Modular design	Modular design
Design	Monotherapy and combinations of divarasib with other anti-cancer therapies	Cohort G (KRAS G12C) ARM A: divarasib ARM B: Docetaxel	ARM E (1L CRC): divarasib + cetuximab + FOLFOX ARM F (2L CRC): divarasib + cetuximab
Primary endpoint	Safety	Progression-free survival	Safety
Status	- FPI Q3 2020 - Data presented at WCLC 2022, ESMO 2022	- BTD granted by FDA Q3 2022 - FPI Q4 2022	FPI Q1 2023
CT Identifier	NCT04449874	NCT03178552	NCT04929223

*Only cohorts with active recruitment shown; NSCLC=Non-small cell lung cancer; WCLC=World Conference on Lung Cancer; ESMO=European Society for Medical Oncology; BTD=Breakthrough therapy designation, CRC=Colorectal cancer

Divarasib (KRAS G12C inhibitor, RG6330, GDC-6036)

A potent, orally available, and selective inhibitor of the KRAS G12C mutant protein

Indication	1L NSCLC
Phase/study	Phase Ib KRASCENDO 170
# of patients	N=60
Design	Combination of divarasib and pembrolizumab in 1L PD-L1+ metastatic NSCLC
Primary endpoint	Safety, tolerability
Status	FPI Q2 2023
CT Identifier	NCT05789082

NSCLC=Non-small cell lung cancer; PD-L1=Programmed cell death-ligand

Crovalimab (RG6107, SKY59)

A humanized monoclonal antibody against complement C5

Indication	Paroxysmal nocturnal hemoglobinuria (PNH)	Paroxysmal nocturnal hemoglobinuria (PNH) patients switching from a C5 inhibitor
Phase/study	Phase I/II COMPOSER	Phase III COMMODORE 1
# of patients	N=59	N=89 (ARMs A/B)
Design	Healthy volunteers and treatment naïve and pretreated patients with PNH: ▪ Part I: Single ascending dose study in healthy subjects ▪ Part II: Intra-patient single ascending dose study in PNH patients ▪ Part III: Multiple-dose study in PNH patients ▪ Part IV: Dose confirmation in PNH patients	▪ ARM A: Crovalimab ▪ ARM B: Eculizumab ▪ ARM C: Patients switching to crovalimab from ravulizumab, higher than labeled doses of eculizumab & C5 SNP patients (descriptive-arm)
Primary endpoint	▪ Safety, PK, PD	▪ Safety
Status	▪ Nonclinical data published in Scientific Reports 2017 Apr; 7(1):1080 ▪ Data presented for Part 2 and 3 at ASH 2018 and 2019	▪ FPI Q3 2020 ▪ Study results in Q1 2023 supported the favorable benefit-risk profile of crovalimab, as seen in the pivotal COMMODORE 2 study ▪ Data presented at EHA 2023 ▪ Filed in US and EU Q2 2023
CT Identifier	NCT03157635	NCT04432584

Crovalimab (RG6107, SKY59)

A humanized monoclonal antibody against complement C5

Indication	Paroxysmal nocturnal hemoglobinuria (PNH) C5 inhibitor naive patients	Paroxysmal nocturnal hemoglobinuria (PNH) C5 inhibitor naive patients (China only)
Phase/study	Phase III COMMODORE 2	Phase III COMMODORE 3
# of patients	N=204	N=51
Design	ARM A: Crovalimab ARM B: Eculizumab	Crovalimab loading dose IV on Day 1, followed by weekly crovalimab SC doses for 4 weeks
Primary endpoint	- Non-inferiority of crovalimab compared to eculizumab: % patients with transfusion avoidance from baseline through week 25 % patients with haemolysis control, as measured by LDH $\leq$ 1.5ULN from week 5-25	- Percentage of patients with transfusion avoidance from baseline through week 25 - Mean percentage of participants with hemolysis control (week 5 through week 25)
Status	- FPI Q4 2020 - Recruitment completed Q2 2022 - Study met its primary endpoint Q1 2023 - Data presented at EHA 2023 - Filed in US and EU Q2 2023	- FPI Q1 2021; Recruitment completed Q3 2021 - Study met its co-primary endpoints Q1 2022 - Filed in China (priority review) Q3 2022 - Data presented at ASH 2022
CT Identifier	NCT04434092	NCT04654468

In collaboration with Chugai

LDH=Lactate Dehydrogenase; ULN=Upper Limit of Normal; IV=Intravenous; SC=Subcutaneous, ASH=American Society of Hematology

Crovalimab (RG6107, SKY59)

A humanized monoclonal antibody against complement C5

Indication	Atypical hemolytic uremic syndrome (aHUS) study 1 - adults	Atypical hemolytic uremic syndrome (aHUS) study 2 - paediatrics
Phase/study	Phase III COMMUTE-a	Phase III COMMUTE-p
# of patients	N=90	N=35
Design	Single-arm study of aHUS patients Cohort 1: not previously treated with C5i Cohort 2: switching from C5i Cohort 3: known C5 polymorphism	Single-arm study of aHUS patients Cohort 1: not previously treated with C5i Cohort 2: switching from C5i ≤18y/o Cohort 3: previously treated with C5i (includes participants with known C5 polymorphism)
Primary endpoint	- Cohort 1+3: proportion of patients with complete TMA response anytime between baseline and week 25 - Cohort 2: proportion of patients with maintained TMA control from baseline through week 25	- Cohort 1: proportion of patients with complete TMA response anytime between baseline and week 25 - Cohort 2: proportion of patients with maintained TMA control from baseline through week 25
Status	FPI Q4 2021	FPI Q4 2021
CT Identifier	NCT04861259	NCT04958265

Crovalimab (RG6107, SKY59)

A humanized monoclonal antibody against complement C5

Indication	Sickle cell disease (SCD) acute treatment	Sickle cell disease (SCD) chronic VOC prevention
Phase/study	Phase Ib CROSSWALK-a	Phase IIa CROSSWALK-c
# of patients	N=30	N=90
Design	- ARM A: Crovalimab - ARM B: Placebo	- ARM A: Crovalimab - ARM B: Placebo
Primary endpoint	Safety	VOC rate, up to 48 weeks
Status	FPI Q1 2022	FPI Q1 2022
CT Identifier	NCT04912869	NCT05075824

VOC=Vaso-occlusive crises

Crovalimab (RG6107, SKY59)

A humanized monoclonal antibody against complement C5

Indication	Lupus nephritis (LN)
Phase/study	Phase I
# of patients	N=15
Design	▪ Single-arm study of patients with active class III, IV, or V lupus nephritis and urine protein-to-creatinine ratio ≥ 1.5 g/g ▪ All patients to receive crovalimab IV loading dose and subsequent crovalimab SC q1w (Day 1, Week 1,2 and 3) followed by corvalimab SC q4w
Primary endpoint	▪ PK, safety
Status	▪ FPI Q1 2023
CT Identifier	ISRCTN12809537

Astegolimab (RG6149, Anti-ST2)

A monoclonal antibody that selective binds to ST2

Indication	Chronic obstructive pulmonary disease (COPD)		
Phase/study	Phase II COPD-ST2OP	Phase IIb ALIENTO	Phase III ARNASA
# of patients	N=81	N=1,290	N=1,290
Design	Astegolimab SC 490mg q4w for 48 weeks	ARM A: SC astegolimab q2w ARM B: SC astegolimab q4w ARM C: SC placebo q2w	ARM A: SC astegolimab q2w ARM B: SC astegolimab q4w ARM C: SC placebo q2w
Primary endpoint	Number of moderate to severe exacerbation	Annualized rate of moderate and severe COPD exacerbations over the 52-week treatment period	Annualized rate of moderate and severe COPD exacerbations over the 52-week treatment period
Status	Published in Lancet Respir Med 2022;10(5):469-477. doi: 10.1016/S2213-2600(21)00556-7	FPI Q4 2021	FPI Q1 2023
CT Identifier	NCT03615040	NCT05037929	NCT05595642

Crenezumab (RG7412)

Humanized monoclonal antibody targeting all forms of Aβ

Indication	Alzheimer’s prevention initiative (API) Colombia
Phase/study	Phase II Cognition study
# of patients	N=252
Design	▪ ARM A: PSEN1 E280A mutation carriers receive crenezumab SC or IV ▪ ARM B: PSEN1 E280A mutation carriers receive placebo ▪ ARM C: non-mutation carriers receive placebo
Primary endpoint	▪ Change on Alzheimer’s Prevention Initiative (API) Composite Cognitive Test total score at 260 weeks treatment ▪ Annualized rate of change in an Episodic Memory Measure: Free and Cued Selective Reminding Task (FCSRT)
Status	▪ Study did not meet its co-primary endpoints Q2 2022 ▪ Data presented at AAIC 2022 ▪ All carriers receive crenezumab
CT Identifier	NCT01998841

Tominersen (RG6042, HTT ASO)

Antisense oligonucleotide (ASO) targeting human HTT mRNA

Indication	Huntington's disease
Phase/study	Phase II GENERATION HD2
# of patients	N=360
Design	Patients aged 25 to 50 years with prodromal (very early subtle signs of HD) or early manifest HD ▪ ARM A: Tominersen 60mg q16w via a lumbar puncture ▪ ARM B: Tominersen 100mg q16w via a lumbar puncture ▪ ARM C: Placebo q16w via a lumbar puncture
Primary endpoint	▪ Safety, biomarkers and efficacy
Status	▪ FPI Q1 2023
CT Identifier	NCT05686551

Fenebrutinib (RG7845, GCD-0853)

Highly selective and reversible (noncovalent) bruton tyrosine kinase

Indication	Primary progressive multiple sclerosis (PPMS)	Relapsing multiple sclerosis (RMS)	
Phase/study	Phase III FENTrepid	Phase III FENhance 1	Phase III FENhance 2
# of patients	N=946	N=736	N=736
Design	ARM A: Fenebrutinib twice daily oral ARM B: Ocrevus 2x300mg IV q24w	ARM A: Fenebrutinib twice daily oral ARM B: Teriflunomide once daily oral	ARM A: Fenebrutinib twice daily oral ARM B: Teriflunomide once daily oral
Primary endpoint	Time to onset of cCDP12	Time to onset of cCDP12 and annualized relapse rate	Time to onset of cCDP12 and annualized relapse rate
Status	FPI Q4 2020	FPI Q1 2021	FPI Q1 2021
CT Identifier	NCT04544449	NCT04586023	NCT04586010

IV=Intravenous; cCDP12=Composite 12-week confirmed disability progression

Fenebrutinib (RG7845, GCD-0853)

Highly selective and reversible (noncovalent) bruton tyrosine kinase

Indication	Relapsing multiple sclerosis (RMS)
Phase/study	Phase II (Biomarker study) FENopta
# of patients	N=109
Design	- ARM A: Fenebrutinib - ARM B: Placebo
Primary endpoint	Total number of new gadolinium-enhancing T1 lesions observed on MRI scans of the brain at 12 weeks
Status	Data presented at EAN 2023
CT Identifier	NCT05119569

Balovaptan (RG7314)

Small molecule antagonist of the V1A vasopressin receptor

Indication	Post-traumatic stress disorder (PTSD)
Phase/study	Phase II
# of patients	N=30
Design	▪ ARM A: Balovaptan IV once a day for 12 weeks ▪ ARM B: Placebo
Primary endpoint	▪ Change from baseline in the Clinician-Administered PTSD Total Symptom Severity Score
Status	▪ FPI Q3 2022
CT Identifier	NCT05401565

PTSD=Post-traumatic stress disorder; IV=Intravenous

Latent myostatin (RG6237, GYM329)

Recycling and antigen-sweeping monoclonal anti-latent myostatin antibody

Indication	Facioscapulohumeral Muscular Dystrophy (FSHD)	Spinal muscular atrophy (SMA)
Phase/study	Phase II MANOEUVRE	Phase II/III MANATEE ¹
# of patients	N=48	N=180
Design	ARM A: 4-week pre-treatment to collect baseline movement data with a wearable device, followed by latent myostatin ARM B: Placebo	ARM A: Part I: GYM329 plus Evrysdi for 24 weeks, followed by GYM329 plus Evrysdi for 72 weeks Part II: GYM329 plus Evrysdi for 72 weeks ARM B: - Placebo plus Evrysdi
Primary endpoint	Percent change in contractile muscle volume of quadriceps femoris muscles by MRI at week 52 and safety	- Change from baseline in RHS score after week 72 of treatment - Safety, PK/PD and muscle biomarkers
Status	FPI Q1 2023	- ODD granted by FDA in Q4 2021 for GYM329 - FPI Part I ambulatory cohort Q2 2022; non-ambulatory cohort July 2023
CT Identifier	NCT05548556	NCT05115110

¹ In collaboration with PTC Therapeutics and SMA Foundation

PK/PD=Pharmacokinetics/Pharmacodynamics; ODD=Orphan drug designation; RHS=Revised hammersmith scale ; MRI=Magnetic Resonance Imaging

Anti-IL-6 (RG6179)

A monoclonal antibody that potently binds interleukin-6 (IL-6) cytokine

Indication	Diabetic macular edema (DME) and Uveitic macular edema (UME)	Diabetic macular edema (DME)	
Phase/study	Phase I DOVETAIL	Phase II BARDENAS	Phase II ALLUVIUM
# of patients	N=90	N=210-230	N=360-400
Design	- Part I: Multiple ascending dose study of intravitreal monotherapy - Part II: monotherapy and in combination with anti-VEGF	ARM A: Anti-IL-6 plus ranibizumab ARM B: Ranibizumab plus sham control	Arm A: 0.25 mg anti-IL-6 q8w Arm B: 1.0 mg anti-IL-6 q8w Arm C: 1.0 mg anti-IL-6 q4w Arm D: 0.5 mg ranibizumab q4w
Primary endpoint	Safety, tolerability, PK	Mean change from baseline in BCVA averaged over week 44 and week 48	Mean change from baseline in BCVA averaged over week 44 and week 48
Status	- FPI Q3 2019 - Data presentation at ARVO 2023	FPI Q4 2021	FPI Q4 2021
CT Identifier		NCT05151744	NCT05151731

Anti-IL-6 (RG6179)

A monoclonal antibody that potently binds interleukin-6 (IL-6) cytokine

Indication	Uveitic macular edema (UME)	
Phase/study	Phase III MEERKAT	Phase III SANDCAT
# of patients	N=225	N=225
Design	ARM A: Anti-IL-6 low-dose q4w to week 12, followed by PRN ARM B: Anti-IL-6 high-dose q4w to week 12, followed by PRN ARM C: Sham control q4w to week 12, followed by PRN	ARM A: Anti-IL-6 low-dose q4w to week 12, followed by PRN ARM B: Anti-IL-6 high-dose q4w to week 12, followed by PRN ARM C: Sham control q4w to week 12, followed by PRN
Primary endpoint	Proportion of participants with ≥ 15 letter improvement from baseline in BCVA at week 16	Proportion of participants with ≥ 15 letter improvement from baseline in BCVA at week 16
Status	FPI Q1 2023	FPI Q1 2023
CT Identifier	NCT05642312	NCT05642325

BCVA=Best corrected visual acuity; PRN= Pro re nata

Roche Group development pipeline

Marketed products development programmes

Roche Pharma global development programmes

Roche Pharma research and early development (pRED)

Genentech research and early development (gRED)

Spark

pRED oncology development programs -1



Molecule	Indication	Phase	# of patients	Status	CT Identifier
Oncology					
FAP-4-1BBL (RG7827)	Solid tumors	I	~150	FPI Q2 2018 Data presented at ESMO 2020 Recruitment completed Q2 2021	
	3L+ MSS mCRC	Ib	80	FPI Q3 2021 Combination study with cibisatamab	NCT04826003
cibisatamab (CEA x CD3, RG7802)	CEA-positive solid tumors	Ia	149	FPI Q4 2014 Data presented at ASCO 2017	NCT02324257
		Ib	228	FPI Q1 2016 Data presented at ASCO 2017	NCT02650713
		Ib	46	FPI Q1 2019	NCT03866239
tobemstomig PD1-LAG3 (RG6139)	Solid tumors	I	320	FPI Q4 2019 Data presented at ESMO 2022 Recruitment completed Q4 2022	NCT04140500
	advanced or metastatic esophageal squamous cell cancer	II	210	FPI Q2 2021 Randomized trial, compared with nivolumab	NCT04785820 TALIOS
	Untreated unresectable or metastatic melanoma	II	80	FPI Q3 2022	NCT05419388
	Non-small cell lung cancer	II	180	FPI Q1 2023	NCT05775289
	advanced and metastatic urothelial cancer	II	240	FPI Q2 2023	NCT05645692
	Metastatic renal cell carcinoma	II	210	FPI Q2 2023	NCT05805501

pRED oncology development programs -2



Molecule	Indication	Phase	# of patients	Status	CT Identifier
Oncology					
englumafusp alfa (CD19-4-1BBL, RG6076)	R/R B cell non-Hodgkin's lymphoma	I	362	Part I: FPI Q3 2019 Part II: FPI Q3 2020 Combination study with Columvi Data presented at ASH 2022 and ICML 2023	NCT04077723
eciskafusp alfa (PD1-IL2v, RG6279)	Solid tumors	Ib	348	Part I: FPI Q2 2020; recruitment completed Q4 2021 Part II: FPI Q1 2022 Part III: FPI Q1 2023	NCT04303858
CD25 (RG6292)	Solid tumors	I	110	FPI Q4 2019 PK/PD data presented at AACR 2023	NCT04158583
forimtamig (Anti-GPRC5D, RG6234)	Multiple myeloma	I	400	FPI Q4 2020 Data presented at EHA 2022 and ASH 2022	NCT04557150
FAP-CD40 (RG6189)	Solid tumors	I	280	FPI Q2 2021	NCT04857138
BRAFi (3) (RG6344)	Solid tumors	I	292	FPI Q1 2022	ISRCTN13713 551
CD19xCD28 (RG6333)	R/R B cell non-Hodgkin's lymphoma	I	~200	FPI Q1 2022 Combination study with Columvi	NCT05219513
EGFRvIIIxCD3 (RG6156)	Glioblastoma	I	~200	FPI Q2 2022	NCT05187624
DLL3 trispecific (RG6524)	Solid tumors	I	168	FPI Q1 2023	NCT05619744
HLA-G CD3 TCB (RG6353)	Solid tumors	I	150	FPI Q2 2023	NCT05769959

pRED neuroscience development programs -1

Molecule	Indication	Phase	# of patients	Status	CT Identifier
Neuroscience					
trontinemab (BS-gantenerumab, RG6102)	Alzheimer's disease	Ila	~120	FPI Q1 2021	NCT04639050
Brain Shuttle-CD20 (BS-CD20, RG6035)	Multiple sclerosis	I	30-63	FPI Q3 2021	ISRCTN16295 177 NCT05704361
ralmitaront (partial TAAR1 agonist, RG7906)	Schizophrenia	II	36	FPI Q4 2018 Recruitment completed Q3 2019	
		II	247	FPI Q4 2019 An interim analysis of TWAIN I did not show evidence of clinical benefit in patients that have negative symptoms of schizophrenia.	NCT03669640 (TWAIN I)
prasinezumab¹ (anti-αSynuclein, RG7935, PRX002)	Parkinson's disease	II	316	The study did not meet its primary endpoint, but showed a reduced clinical decline of core motor signs (MDS UPDRS partIII). Data presented at MDS & ADPD 2020-22. The Open Label Extension is ongoing.	NCT03100149 (PASADENA)
		IIb	575	FPI Q2 2021 Recruitment completed Q1 2023	NCT04777331 (PADOVA)
alogabat (GABA-Aα5 PAM, RG7816)	Autism spectrum disorder	II	105	FPI Q1 2021	NCT04299464 (Aurora)

pRED neuroscience development programs -2

Molecule	Indication	Phase	# of patients	Status	CT Identifier
Neuroscience					
rugonersen (UBE3A LNA, RG6091)	Angelman syndrome	I	66	FPI Q3 2020	NCT04428281
MAGLi (RG6182)	Multiple sclerosis	I	Up to 36	FPI July 2023	
NME (RG6289)	Alzheimer's disease	I	138	FPI Q4 2021	
NME (RG6163)	Psychiatric disorders	I	84	FPI Q1 2022	
selnoflast* (NLRP3i, RG6418)	Parkinson's disease	Ib	48	FPI Q3 2022	
basmisamil (GABA-Aa5 NAM, RG1662)	Dup15q syndrome	II	90	FPI Q4 2022	NCT05307679

*molecule also in gRED development: Phase Ic in coronary artery disease with FPI Q4 2022

pRED immunology and ophthalmology development programs

Molecule	Indication	Phase	# of patients	Status	CT Identifier
Immunology					
selnoflast* (NLRP3i, RG6418)	Chronic obstructive pulmonary disease	Ib	102	FPI Q2 2022 Study closed Q3 2022	
Ophthalmology					
zifibancimig (VEGF-Ang2 DutaFab, RG6120)	nAMD	I	251	FPI Q4 2020	NCT04567303 (BURGUNDY)
vicasinabin (CB2 receptor agonist, RG7774)	DR	II	135	FPI Q2 2020 Recruitment completed Q3 2022	NCT04265261 (CANBERRA)
NME (RG6209)	retinal disease	I	~70 (Part I)	FPI Q4 2022	

*molecule also in gRED development: Phase Ic in coronary artery disease with FPI Q4 2022

pRED infectious diseases development programs

Molecule	Indication	Phase	# of patients	Status	CT Identifier
Infectious Diseases					
ruzotolimod (TLR7 agonist (3) RG7854)	Chronic hepatitis B	I	150	FPI Q4 2016 Data presented at APASL 2019	NCT02956850
ruzotolimod/ xalnesiran/ PDL1 LNA (RG7854/RG6346/RG6084)	Chronic hepatitis B	II	275	FPI Q3 2020	NCT04225715 (PIRANGA)
PDL1 LNA (RG6084)	Chronic hepatitis B	I	35	FPI Q1 2019 Part Ia: completed Part Ib: initiated	
zosurabalpin (Abx MCP, RG6006)	A. baumannii infections	I	204	FPI Q4 2020	NCT04605718
HBsAg MAb (RG6449)	Chronic hepatitis B	I	110	FPI Q2 2023	NCT05763576

Roche Group development pipeline

Marketed products development programmes

Roche Pharma global development programmes

Roche Pharma research and early development (pRED)

Genentech research and early development (gRED)

Spark

gRED oncology development programs -1

Molecule	Indication	Phase	# of patients	Status	CT Identifier
Oncology					
cevostamab (anti-FcRH5 x CD3; RG6160)	R/R multiple myeloma	I	300	FPI Q3 2017 Data presented at ASH 2020, 2021 & 2022	NCT03275103
	R/R multiple myeloma	I	120	FPI Q2 2021	NCT04910568
	BCMA-experienced R/R MM	I/II	140	FPI Q4 2022	NCT05535244
runimotamab (HER2 x CD3, RG6194)	Metastatic HER2-expressing cancers	I	440	FPI Q2 2018	NCT03448042
NME (RG6286)	Locally advanced or metastatic colorectal cancer	I	67	FPI Q3 2020	NCT04468607
IL 15/IL 15Ra-Fc (RG6323)¹	Solid tumors	Ia/Ib	250	FPI Q1 2020	NCT04250155
	R/R multiple myeloma	I	60	FPI Q2 2022	NCT05243342
	R/R multiple myeloma	I	90	FPI Q1 2023 Combination study with cevostamab	NCT05646836
autogene cevumeran (Individualized Neoantigen-Specific Therapy (iNeST); RG6180) ²	Solid tumors	Ia/Ib	272	FPI Q4 2017 Data presented at AACR 2020 Recruitment completed Q1 2022	NCT03289962
	1L advanced melanoma	II	131	FPI Q1 2019	NCT03815058 (IMcode001)

gRED oncology development programs -2

Molecule	Indication	Phase	# of patients	Status	CT Identifier
Oncology					
SHP2i (RG6433) ¹	Solid tumors	Ib	~125	FPI Q3 2022	NCT05487235
	KRAS-G12C mutant solid tumors	Ib	~500	FPI Q4 2021 Arm F of a combination study investigating divarasil monotherapy and combinations	NCT04449874
belvarafenib (RG6185) ²	nRASmt CPI-experienced melanoma	Ib	83	FPI Q2 2021 Data presented at ESMO 2021	NCT04835805
NME (RG6411)	Solid tumors	I	110	FPI Q4 2022	NCT05581004
AR degrader (RG6537) ³	mCRPC	I	~160	FPI Q2 2023	NCT05800665

gRED immunology and ophthalmology development programs

Molecule	Indication	Phase	# of patients	Status	CT Identifier
Immunology					
NME (RG6287, GDC-8264)	Acute graft versus host disease	Ib	40	FPI Q2 2023	NCT05673876
NME (RG6315, MTBT1466A)	Immunologic disorders	I	~24	FPI Q3 2020 Recruitment completed Q2 2022	
	Systemic sclerosis	Ib	100	FPI Q1 2023	NCT05462522
NME (RG6341, GDC-6599)	Asthma	Ia/Ib	84	FPI Q4 2021	
	Chronic cough	Ila	80	FPI Q1 2023	NCT05660850
TMEM16A potentiator (RG6421, GDC-6988)	Cystic fibrosis	Ib	30	FPI Q3 2022 Recruitment completed Q2 2023	ISRCTN15406 513
Vixarelimab (RG6536)¹	Idiopathic pulmonary fibrosis / Systemic sclerosis-associated interstitial lung disease	II	~290	FPI Q2 2023	NCT05785624

Ophthalmology					
NME (RG6351)	DME	I	42-78	FPI Q2 2022	ISRCTN14152 148
OpRegen (RG6501)²	Geographic atrophy	II	60	FPI Q1 2023	NCT05626114

gRED neuroscience and infectious diseases development programs



Molecule	Indication	Phase	# of patients	Status	CT Identifier
Neuroscience					
semorinemab (RG6100)¹	Mild-to-moderate Alzheimer's disease	II	272	FPI Q1 2019 One of two co-primary endpoints met Q3 2021 Data presented at CTAD 2021 The Open Label Extension is ongoing	NCT03828747 (LAURIET)
Infectious Diseases					
LepB inhibitor (RG6319)	Complicated urinary tract infection	I	32	FPI Q1 2023	

Roche Group development pipeline

Marketed products development programmes

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Spark

Hemophilia A

Unique gene therapy platform

Molecule	Dirloctogene Samoparvovec (SPK-8011) (RG6357)	
Indication	Hemophilia A	
Phase/study	Phase I	Phase I/II
# of patients	N=100	N=30
Design	Long term follow up study of patients who have received SPK-8011 in any prior Spark-sponsored SPK-8011 study	Gene transfer, dose-finding safety, tolerability, and efficacy study of SPK-8011
Primary endpoint	Safety	Safety and changes from baseline in FVIII activity levels at week 52
Status	Ongoing	- Updated data presented at ISTH 2020 and 2021 - Recruitment completed Q1 2021 - Data published in <i>NEJM</i> 2021; 385:1961-1973 5-year data published at ASH 2022
CT Identifier	NCT03432520	NCT03003533

Pompe disease

Unique gene therapy platform

Molecule	SPK-3006 (RG6359)
Indication	Pompe disease
Phase/study	Phase I/II RESOLUTE
# of patients	N=20
Design	Gene transfer study for late-onset Pompe disease
Primary endpoint	Safety
Status	- FPI Q4 2020 - Recruitment completed Q2 2022
CT Identifier	NCT04093349

Doing now what patients need next