

Selected development projects

Compound/ product	Common name	Mechanism of action	Potential indication	Business franchise	Formulation/ route of administration	Year project entered current development phase	Planned filing dates/current phase
ABL001	asciminib	BCR-ABL inhibitor	Chronic myeloid leukemia, 3 rd line	Oncology	Oral	2017	2021/III
ACZ885	canakinumab	IL-1 beta inhibitor	Non-small cell lung cancer, 2 nd line	Oncology	Subcutaneous injection	2019	2021/III
			Non-small cell lung cancer, 1 st line	Oncology	Subcutaneous injection	2018	2021/III
			Non-small cell lung cancer, adjuvant	Oncology	Subcutaneous injection	2018	2023/III
AVXS-101 (OAV101)	onasemnogene abeparvovec	Survival motor neuron (SMN) gene therapy	Spinal muscular atrophy (IT formulation) ¹	Neuroscience	Intrathecal injection	2018	TBC based on FDA feedback/I/II
AVXS-201 (OAV201)	TBD	Methyl-CpG binding protein 2 (MECP2) gene therapy	Rett syndrome	Neuroscience	Intrathecal injection	2018	≥2025/I
Beovu	brolocizumab	VEGF inhibitor	Diabetic macular edema	Ophthalmology	Intravitreal injection	2018	2021/III
			Retinal vein occlusion	Ophthalmology	Intravitreal injection	2019	2023/III
			Diabetic retinopathy ²	Ophthalmology	Intravitreal injection	2020	2023/III
BYL719	alpelisib	PI3K-alpha inhibitor	PIK3CA-related overgrowth spectrum	Oncology	Oral	2020	2021/II
			Triple negative breast cancer	Oncology	Oral	2020	2023/III
			Human epidermal growth factor receptor 2-positive (HER2+) advanced breast cancer ³	Oncology	Oral	2020	≥2025/III
			Ovarian cancer	Oncology	Oral	2019 ⁴	2023/III
			Head and neck squamous cell carcinoma, 2 nd and 3 rd line ⁵	Oncology	Oral	2019 ⁶	≥2025/III
CEE321	TBD	Pan-JAK inhibitor	Atopic dermatitis	Immunology, Hepatology and Dermatology	Topical	2019 ⁷	≥2025/I
CFZ533 ⁸	iscalimab	CD40 inhibitor	Renal transplantation	Immunology, Hepatology and Dermatology	Intravenous infusion	2018	≥2025/II
			Liver transplantation	Immunology, Hepatology and Dermatology	Intravenous infusion	2019	≥2025/II
			Sjögren's syndrome	Immunology, Hepatology and Dermatology	Intravenous infusion	2019	≥2025/II
Coartem	artemether + lumefantrine	PGH-1	Malaria, uncomplicated (<5 kg patients) ⁹	Established Medicines	Oral	2020	2024/III
Cosentyx	secukinumab	IL-17A inhibitor	Ankylosing spondylitis head-to-head study versus Sandoz biosimilar Hyrimoz (adalimumab)	Immunology, Hepatology and Dermatology	Subcutaneous injection	2017	2022/III
			Hidradenitis suppurativa	Immunology, Hepatology and Dermatology	Subcutaneous injection	2019	2022/III
			Giant cell arteritis	Immunology, Hepatology and Dermatology	Subcutaneous injection	2019	2024/II
			Lichen planus	Immunology, Hepatology and Dermatology	Subcutaneous injection	2020	≥2025/II
			Lupus nephritis ¹⁰	Immunology, Hepatology and Dermatology	Subcutaneous injection	2020	≥2025/III
			Psoriatic arthritis (IV formulation) ¹¹	Immunology, Hepatology and Dermatology	Intravenous infusion	2019	2022/III
			Ankylosing spondylitis (IV formulation) ¹²	Immunology, Hepatology and Dermatology	Intravenous infusion	2019	2023/III
CSJ117	TBD	TSLP inhibitor	Asthma ¹³	Respiratory	Inhalation	2020	≥2025/II
ECF843	TBD	rh-Lubricin	Dry eye	Ophthalmology	Topical	2020	2023/II

¹ Preclinical studies to address partial clinical hold are on track. The FDA has acknowledged the potential of AVXS-101 IT in this patient population and recommends a pivotal confirmatory study, to be initiated after partial clinical hold is lifted.

² Previously disclosed as proliferative diabetic retinopathy

³ Previously disclosed as hormone receptor-negative (HR-)/human epidermal growth factor receptor 2-positive (HER2+) advanced breast cancer

⁴ Reflects the year in which the decision to enter the disclosed phase was made; "first patient, first visit" has not yet occurred

⁵ Previously disclosed as head and neck squamous cell carcinoma

⁶ Reflects the year in which the decision to enter the disclosed phase was made; "first patient, first visit" has not yet occurred

⁷ Reflects the year in which the decision to enter the disclosed phase was made; "first patient, first visit" has not yet occurred

⁸ The renal transplantation and liver transplantation indications were previously disclosed as solid organ transplantation. This has since split into two separate projects.

⁹ Project added to selected development projects table in 2020 - entered Confirmatory Development

¹⁰ Project added to selected development projects table in 2020 - entered Confirmatory Development

¹¹ Project added to selected development projects table in 2020 - in Confirmatory Development

¹² Project added to selected development projects table in 2020 - in Confirmatory Development

¹³ Previously disclosed as severe asthma

Item 4. Information on the Company

Compound/ product	Common name	Mechanism of action	Potential indication	Business franchise	Formulation/ route of administration	Year project entered current development phase	Planned filing dates/current phase
Entresto	valsartan and sacubitril (as sodium salt complex)	Angiotensin receptor/ neprilysin inhibitor	Chronic heart failure with preserved ejection fraction	Cardiovascular, Renal and Metabolism	Oral	2020	US registration
			Post-acute myocardial infarction	Cardiovascular, Renal and Metabolism	Oral	2016	2021/III
Jakavi	ruxolitinib	JAK1/2 inhibitor	Acute graft-versus-host disease	Oncology	Oral	2017	2021/III
			Chronic graft-versus-host disease	Oncology	Oral	2017	2021/III
KAE609	cipargamin	PfATP4 inhibitor	Malaria, uncomplicated ¹⁴	Established Medicines	Oral	2017	≥2025/II
			Malaria, severe ¹⁵	Established Medicines	Oral	2019 ¹⁶	≥2025/II
KAF156	ganaplacide	Imidazolopiperazines derivative	Malaria, uncomplicated ¹⁷	Established Medicines	Oral	2017	≥2025/II
Kisqali	ribociclib	CDK4 inhibitor	Hormone receptor-positive (HR+)/human epidermal growth factor receptor 2-negative (HER2-) early breast cancer (adjuvant) ¹⁸	Oncology	Oral	2018	2023/III
KJX839 ¹⁹	inclisiran	siRNA (regulation of LDL-C)	Hyperlipidemia	Cardiovascular, Renal and Metabolism	Subcutaneous injection	2020	EU approved US ²⁰
			Secondary prevention of cardiovascular events in patients with elevated levels of LDL-C	Cardiovascular, Renal and Metabolism	Subcutaneous injection	2018	≥2025/III
Kymriah	tisagen- lecleucel	CD19 CAR-T	Relapsed/refractory follicular lymphoma	Oncology	Intravenous infusion	2018	2021/II
			Relapsed/refractory diffuse large B-cell lymphoma in 1 st relapse	Oncology	Intravenous infusion	2019	2021/III
LJC242	tropifexor, cenicriviroc (in fixed-dose combination)	FXR agonist and CCR2 inhibitor	Nonalcoholic steatohepatitis	Immunology, Hepatology and Dermatology	Oral	2018	≥2025/II
LJN452	tropifexor, licogliflozin (in fixed-dose combination)	FXR agonist and SGLT1/2 inhibitor	Nonalcoholic steatohepatitis	Immunology, Hepatology and Dermatology	Oral	2019	≥2025/II
LMI070	branaplam	SMN2 RNA splicing modulator	Spinal muscular atrophy	Neuroscience	Oral	2015	≥2025/II
LNP023	iptacopan	CFB inhibitor	IgA nephropathy	Cardiovascular, Renal and Metabolism	Oral	2018	2023/II
			C3 glomerulopathy	Cardiovascular, Renal and Metabolism	Oral	2019	2023/II
			Paroxysmal nocturnal hemoglobinuria	Cardiovascular, Renal and Metabolism	Oral	2018	2023/II
			Membranous nephropathy	Cardiovascular, Renal and Metabolism	Oral	2019	≥2025/II
LOU064	remibrutinib	BTK inhibitor	Chronic spontaneous urticaria	Immunology, Hepatology and Dermatology	Oral	2019	≥2025/II
			Sjögren's syndrome ²¹	Immunology, Hepatology and Dermatology	Oral	2019	≥2025/II
Lutathera	lutetium Lu 177 dotatate/ lutetium (¹⁷⁷ Lu) oxodotreotide	Radioligand therapy targeting SSTR	Gastroenteropancreatic neuroendocrine tumors, 1 st line in G2/3 tumors ²²	Oncology	Intravenous infusion	2020	2023/III

¹⁴ Previously disclosed as malaria

¹⁵ Previously disclosed as severe malaria

¹⁶ Reflects the year in which the decision to enter the disclosed phase was made; "first patient, first visit" has not yet occurred

¹⁷ Previously disclosed as malaria

¹⁸ Previously disclosed as HR+/HER2- breast cancer (adjuvant)

¹⁹ Approved in the EU as *Leqvio* for primary hypercholesterolemia and mixed dyslipidemia

²⁰ Novartis received a complete response letter (CRL) from the FDA due to unresolved facility inspection-related conditions at a third-party manufacturing facility in Europe. The FDA has not raised any concerns related to the efficacy or safety of inclisiran. A response to the CRL is planned to be submitted in Q2-Q3 2021.

²¹ Project added to selected development projects table in 2020 - in Confirmatory Development

²² Project added to selected development projects table in 2020 - entered Confirmatory Development

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¹⁷⁷ Lu- PSMA-617	TBD	Radiorigand therapy targeting PSMA	Metastatic castration-resistant prostate cancer	Oncology	Intravenous infusion	2018	2021/III
LXE408	TBD	Protozoane inhibitor	Visceral leishmaniasis	Established Medicines	Oral	2019 ²³	≥2025/II
MBG453	sabatolimab	TIM-3 antagonist	Myelodysplastic syndrome	Oncology	Intravenous infusion	2020	2021/III
			Unfit acute myeloid leukemia ²⁴	Oncology	Intravenous infusion	2020	2024/II
OMB157 ²⁵	ofatumumab	Anti-CD20 monoclonal antibody	Relapsing multiple sclerosis	Neuroscience	Subcutaneous injection	2020	US approved EU registration
PDR001	spartalizumab	PD-1 inhibitor	Malignant melanoma (combo) ²⁶	Oncology	Intravenous infusion	2018	≥2025/II
QBW251	icenticaftor	CFTR potentiator	Chronic obstructive pulmonary disease	Respiratory	Oral	2019	2024/II
QGE031	ligelizumab	IgE inhibitor	Chronic spontaneous urticaria ²⁷	Immunology, Hepatology and Dermatology	Subcutaneous injection	2018	2022/III
SAF312	TBD	TRPV1 antagonist	Chronic ocular surface pain	Ophthalmology	Topical	2016	2024/II
<i>Tabrecta</i>	capmatinib	c-MET inhibitor	Solid tumors	Oncology	Oral	2019 ²⁸	2024/II
TQJ230	pelacarsen	ASO targeting Lp(a)	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein(a)	Cardiovascular, Renal and Metabolism	Subcutaneous injection	2019	≥2025/III
UNR844	TBD	Reduction of disulfide bonds	Presbyopia	Ophthalmology	Topical	2019	2024/II
VAY736	ianalumab	BAFF-R inhibitor	Autoimmune hepatitis	Immunology, Hepatology and Dermatology	Subcutaneous injection	2018	≥2025/II
			Sjögren's syndrome ²⁹	Immunology, Hepatology and Dermatology	Subcutaneous injection	2017	≥2025/II
VPM087	gevokizumab	IL-1 beta antagonist	Colorectal cancer, 1 st line	Oncology	Intravenous infusion	2019	≥2025/I
<i>Xolair</i>	omalizumab	IgE inhibitor	Food allergy	Respiratory	Subcutaneous injection	2019	2022/III

²³ Reflects the year in which the decision to enter the disclosed phase was made; "first patient, first visit" has not yet occurred

²⁴ Previously disclosed as acute myeloid leukemia

²⁵ Approved in the US as *Kesimpta* for relapsing multiple sclerosis

²⁶ Previously disclosed as metastatic melanoma (combo)

²⁷ Previously disclosed as chronic spontaneous urticaria/chronic idiopathic urticaria

²⁸ Reflects the year in which the decision to enter the disclosed phase was made; "first patient, first visit" has not yet occurred

²⁹ Previously disclosed as primary Sjögren's syndrome

Projects removed from the development table since 2019

Compound/ product	Potential indication	Change	Reason
AVXS-101	Spinal muscular atrophy (IV formulation)	Commercialized as <i>Zolgensma</i>	
BYL719	PIK3CA mutant hormone receptor-positive (HR+)/human epidermal growth factor receptor 2-negative (HER2-) postmenopausal advanced breast cancer, 2 nd line (+ fulvestrant)	Commercialized as <i>Piqray</i>	
<i>Cosentyx</i>	Non-radiographic axial spondyloarthritis	Commercialized	
	Psoriatic arthritis head-to-head study versus Humira® (adalimumab)	Publication achieved	
INC280	Non-small cell lung cancer	Commercialized as <i>Tabrecta</i>	
<i>Kymriah</i>	Relapsed/refractory diffuse large B-cell lymphoma (+ pembrolizumab)	Removed	Development discontinued
LAM320	Multidrug-resistant tuberculosis	Removed	Planned US submission discontinued
PDR001	Metastatic BRAF V600+ melanoma (w/ <i>Tafinlar</i> + <i>Mekinist</i>)	Removed	Development discontinued
QMF149	Asthma	Commercialized as <i>Ateectura Breezhaler</i>	
QVM149	Asthma	Commercialized as <i>Energair Breezhaler</i>	
RTH258	Neovascular (wet) age-related macular degeneration	Commercialized as <i>Beovu</i>	
SEG101	Sickle cell disease	Commercialized as <i>Adakveo</i>	
VPM087	Renal cell carcinoma, 1 st line	Removed	Development discontinued
<i>Xolair</i>	Nasal polyps	Commercialized	
ZPL389	Atopic dermatitis	Removed	Development discontinued