Pipeline

Novartis is consistently rated as having one of the industry's most respected development pipelines, with more than 200 projects in clinical development, as of December 31, 2017.

Many of these projects, which include new molecular entities as well as additional indications and different formulations for marketed products, are for potentially best-in-class or first-in-class medicines that could significantly advance treatment standards for patients worldwide. This table provides an overview of selected projects in confirmatory development, organized according to our development units.

We use the traditional pipeline model as a platform (e.g., Phase I-III). However, we have tailored the process to be simpler, more flexible and more efficient.

Glossary

Project/product Project refers to the Novartis reference code (combination of three letters and three numbers) used for projects in development. Product refers to the brand name for a marketed product.

Common name Official international non-proprietary name or generic name for an individual molecular entity as designated by the World Health Organization

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Major development projects

Project/product	Common name	Mechanism of action	Potential indication/disease area	Route of administration	Planned filing dates [1,2]	PHASE I	PHASE II	PHASE III	SUBMISSION
Oncology									
MTV273	–	BCMA-targeted chimeric antigen receptor T-cell immunotherapy	Multiple myeloma	Intravenous infusion	2021				
HDM201	–	p53-HDM2 inhibitor	Acute myeloid lymphoma	Oral	≥2022				
INC280	capmatinib	c-MET inhibitor	Non-small cell lung cancer (NSCLC) [lead indication]; NSCLC (EGFR mutation)	Oral	2019				
ABL001	asciminib	BCR-ABL inhibitor	Chronic myeloid leukemia (CML) [lead indication], 3rd line; CML, 1st line	Oral	2020				
ACZ885	canakinumab	Anti-interleukin-1 beta monoclonal antibody	NSCLC, 2nd line; NSCLC, 1st line; adjuvant NSCLC	Subcutaneous injection	2021				
EGF816	–	EGFR mutation modulation	NSCLC	Oral	2020				
BYL719	alpelisib	PI3K-alpha inhibitor	Hormone receptor-positive (HR+)/human epidermal growth factor receptor 2-negative (HER2-) advanced breast cancer (postmenopausal women), 2nd line (+ fulvestrant)	Oral	2018				
Jakavi	ruxolitinib	JAK1/2 inhibitor	Acute graft-versus-host disease; chronic graft-versus-host disease	Oral	2020				
LCI699	osilodrostat	Cortisol synthesis inhibitor	Cushing's disease	Oral	2018				
Promacta/Revolade	eltrombopag	Thrombopoietin receptor agonist	Severe aplastic anemia, 1st line	Oral	2018				
SEG101	crizanlizumab	P-selectin inhibitor	Sickle cell disease	Intravenous infusion	2019				
Arzerra	ofatumumab	Anti-CD20 monoclonal antibody	Refractory indolent non-Hodgkin's lymphoma	Intravenous infusion	2020				
PDR001	spartalizumab	Anti-PD-1 monoclonal antibody	Malignant melanoma (*Tafinlar + Mekinist*) [lead indication]; malignant melanoma; endocrine neoplasm	Intravenous infusion	2019				
Rydapt	midostaurin	Signal transduction inhibitor	Acute myeloid leukemia (FLT3 wild type)	Oral	≥2022				
Kisqali	ribociclib	CDK4/6 inhibitor	HR+/HER2- advanced breast cancer (postmenopausal women), 1st/2nd line (+ fulvestrant); HR+/HER2- advanced breast cancer (premenopausal women), 1st line (+ tamoxifen + goserelin or NSAI[3] + goserelin); HR+/HER2- breast cancer (adjuvant)	Oral	2018				
Tafinlar + Mekinist	dabrafenib + trametinib	BRAF inhibitor + MEK inhibitor	BRAF V600+ melanoma (adjuvant)	Oral	US/EU registration				
CTL019[4]	tisagenlecleucel	CD19-targeted chimeric antigen receptor T-cell immunotherapy	Pediatric/young adult acute lymphoblastic leukemia [lead indication]; r/r diffuse large B-cell lymphoma; r/r follicular lymphoma; chronic lymphocytic leukemia; r/r diffuse large B-cell lymphoma (+ pembrolizumab); r/r diffuse large B-cell lymphoma in 1st relapse	Intravenous infusion	US approved EU registration				
Afinitor/Votubia	everolimus	mTOR inhibitor	Tuberous sclerosis complex seizures	Oral	EU approved US registration				
Signifor LAR	pasireotide	Somatostatin analogue	Cushing's disease	Long-acting release/ intramuscular injection	EU approved US registration				

[1] Some filings have received approval in either the US or EU but are awaiting approval in the other market.
[2] Phase and planned filing dates refer to the lead indication in development.
[3] Non-steroidal aromatase inhibitor
[4] Approved in the US as *Kymriah*

Pipeline (continued)

Mechanism of action Specific biochemical interaction with a molecular target such as a receptor or enzyme, through which a drug substance produces its pharmacological effect

Potential indication/indications Disease or condition for which a compound or marketed product is in development and is being studied as a potential therapy

Route of administration Path by which a medicinal preparation is administered into the body, such as oral, subcutaneous or intravenous

Phase I First clinical trials of a new compound, generally performed in a small number of healthy human volunteers, to assess the clinical safety and tolerability, as well as metabolic and pharmacologic properties of the compound

Phase II Clinical studies with patients who have the target disease, with the aim of continuing the Phase I safety assessment in a larger group, assessing the efficacy of the drug in the patient population, and determining the appropriate doses for further evaluation

Phase III Large-scale clinical studies with several hundred to several thousand patients, which are conducted to establish the safety and efficacy of the drug in specific indications for regulatory approval. Phase III trials also may be used to compare a new drug against a current standard of care to evaluate the overall benefit-risk relationship of the new medicine.

Glossary continued on page 62

Major development projects

Project/product	Common name	Mechanism of action	Potential indication/disease area	Route of administration	Planned filing dates[1,2]	PHASE I	PHASE II	PHASE III	SUBMISSION
Cardiovascular and metabolism									
LHW090	–	Neprilysin inhibitor	Resistant hypertension	Oral	≥2022				
LIK066	–	SGLT1/2 inhibitor	Weight loss	Oral	≥2022				
MAA868	–	Factor XI inhibitor	Stroke prevention; atrial fibrillation	Subcutaneous injection	≥2022				
Entresto	valsartan, sacubitril (as sodium salt complex)	Angiotensin receptor/neprilysin inhibitor	Chronic heart failure with preserved ejection fraction [lead indication]; post-acute myocardial infarction	Oral	2019				
ACZ885	canakinumab	Anti-interleukin-1 beta monoclonal antibody	Secondary prevention of cardiovascular events	Subcutaneous injection	US/EU registration[5]				
Respiratory									
QBW251	–	CFTR potentiator	Chronic obstructive pulmonary disease	Oral	≥2022				
QMF149	indacaterol, mometasone furoate (in fixed-dose combination)	Long-acting beta2-adrenergic agonist and inhaled corticosteroid	Asthma	Inhalation	2019				
QAW039	fevipiprant	DP2 antagonist (CRTH2 antagonist)	Asthma	Oral	2020				
Xolair	omalizumab	Anti-IgE monoclonal antibody	Nasal polyps	Subcutaneous injection	2020				
QVM149	indacaterol, mometasone furoate, glycopyrronium bromide (in fixed-dose combination)	Long-acting beta2-adrenergic agonist, long-acting muscarinic antagonist and inhaled corticosteroid	Asthma	Inhalation	2019				
Immunology and dermatology									
LJN452	tropifexor	FXR agonist	Nonalcoholic steatohepatitis	Oral	≥2022				
VAY736	–	Anti-BAFF (B-cell-activating factor) monoclonal antibody	Autoimmune hepatitis [lead indication]; primary Sjögren's syndrome	Subcutaneous injection	2021				
VAY785	emricasan	Pan-caspase inhibitor	Nonalcoholic steatohepatitis	Oral	≥2022				
CFZ533	–	Blocking, non-depleting, anti-CD40 monoclonal antibody	Solid organ transplantation	Intravenous infusion	≥2022				
LOU064	–	BTK inhibitor	Chronic spontaneous urticaria	Oral	≥2022				
ZPL389	–	Histamine H4 receptor antagonist	Atopic dermatitis	Oral	2021				
QGE031	ligelizumab	High-affinity anti-IgE monoclonal antibody	Chronic spontaneous urticaria; chronic idiopathic urticaria	Subcutaneous injection	2021				
Cosentyx	secukinumab	Anti-interleukin-17 monoclonal antibody	Non-radiographic axial spondyloarthritis; psoriatic arthritis head-to-head study versus adalimumab; ankylosing spondylitis head-to-head study versus proposed Sandoz biosimilar adalimumab	Subcutaneous injection	2019				
Neuroscience									
EMA401	olodanrigan	Angiotensin II type 2 receptor antagonist	Peripheral neuropathic pain	Oral	2021				
BYM338	bimagrumab	Inhibitor of activin type 2 receptor	Hip fracture recovery [lead indication]; sarcopenia	Intravenous infusion	≥2022				
CAD106	amilomotide	Beta-amyloid-protein therapy	Alzheimer's disease	Intramuscular injection	≥2022				
CNP520	–	BACE inhibitor	Alzheimer's disease	Oral	≥2022				
BAF312	siponimod	Sphingosine-1-phosphate receptor modulator	Secondary progressive multiple sclerosis	Oral	2018				
LMI070	branaplam	SMN2 RNA splicing modulator	Spinal muscular atrophy	Oral	2021				
OMB157	ofatumumab	Anti-CD20 monoclonal antibody	Relapsing multiple sclerosis	Subcutaneous injection	2019				
Gilenya	fingolimod	Sphingosine-1-phosphate receptor modulator	Pediatric multiple sclerosis	Oral	US/EU registration				
AMG 334	erenumab	Selective CGRP receptor antagonist	Prophylaxis of migraine	Subcutaneous injection	US/EU registration				

[1] Some filings have received approval in either the US or EU but are awaiting approval in the other market.
[2] Phase and planned filing dates refer to the lead indication in development.
[5] Submission pending acceptance by the FDA and EMA

Pipeline (continued)

Advanced development Medical device project for which a positive proof of concept has been established, and clinical and non-clinical studies are being conducted to establish the device's safety, efficacy or performance. This is needed to address regulatory requirements for obtaining marketing authorization.

Submission Application for marketing approval has already been submitted to one or both of the following regulatory agencies: the US Food and Drug Administration (FDA), the European Medicines Agency (EMA). Novartis has not yet received marketing authorization from both regulatory agencies. The application contains comprehensive data and information gathered during human clinical trials and animal studies conducted through the various phases of drug development.

Major development projects

Project/product	Common name	Mechanism of action	Potential indication/disease area	Route of administration	Planned filing dates [1,2]	PHASE I	PHASE II	PHASE III	SUBMISSION
Infectious diseases									
KAF156	–	Imidazolopiperazines derivative	Malaria	Oral	≥2022				
KAE609	cipargamin	PfATP4 inhibitor	Malaria	Oral	≥2022				
LAM320	clofazimine	Mycobacterial DNA binding	Multidrug-resistant tuberculosis	Oral	2018				
Ophthalmology									
ECF843	–	Boundary lubricant	Dry eye	Eye drops	≥2022				
UNR844	–	Reduction of disulfide bonds	Presbyopia	Eye drops	2021				
RTH258	brolucizumab	Anti-vascular endothelial growth factor (VEGF) single-chain antibody fragment	Neovascular age-related macular degeneration [lead indication]; diabetic macular edema	Intravitreal injection	2018				
Lucentis	ranibizumab	Anti-VEGF monoclonal antibody fragment	Retinopathy of prematurity	Intravitreal injection	2018				
Clareon IOL with *AutonoMe* pre-loaded delivery device	–	N/A	Next-generation IOL	Cataract implant	US 2019	ADVANCED DEVELOPMENT			
AcrySof IQ PanOptix IOL	–	N/A	Trifocal IOL	Cataract implant	US 2019	ADVANCED DEVELOPMENT			
AcrySof IQ PanOptix Toric IOL	–	N/A	Trifocal IOL for astigmatism	Cataract implant	US 2019	ADVANCED DEVELOPMENT			
A02062	–	N/A	Extended depth of focus IOL	Cataract implant	US 2019 EU 2019	ADVANCED DEVELOPMENT			
A02238	–	N/A	Mid-tier phacoemulsification device	Cataract equipment	US 2018 EU 2018	ADVANCED DEVELOPMENT			
A02972	–	N/A	Digital visualization system connected with *Constellation*	Vitreoretinal equipment	US 2018 EU 2018	ADVANCED DEVELOPMENT			
A02491	–	N/A	New monthly disposable lens	Vision care	US 2020 EU 2020	ADVANCED DEVELOPMENT			
A02931	–	N/A	New weekly disposable lens	Vision care	US 2020 EU 2020	ADVANCED DEVELOPMENT			
A00717	–	N/A	Daily disposable line extension	Vision care	EU 2018	ADVANCED DEVELOPMENT			
A01660	–	N/A	New daily disposable lens	Vision care	US 2018 EU 2018	ADVANCED DEVELOPMENT			
Biosimilars									
GP1111	infliximab	TNF-alpha inhibitor	Inflammatory bowel disease; rheumatoid arthritis; plaque psoriasis (same as originator)	Intravenous	EU registration				
GP2017	adalimumab	TNF-alpha inhibitor	Arthritides (rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis); plaque psoriasis and others (same as originator)	Subcutaneous	US/EU registration				
GP2013	rituximab	Anti-CD20 monoclonal antibody	Non-Hodgkin's lymphoma; chronic lymphocytic leukemia; rheumatoid arthritis; granulomatosis with polyangiitis; microscopic polyangiitis (same as originator)	Intravenous	EU approved US registration				
LA-EP2006	pegfilgrastim	Pegylated granulocyte colony-stimulating factor	Chemotherapy-induced neutropenia and others (same as originator)	Subcutaneous	EU registration US 2019[6]				

[1] Some filings have received approval in either the US or EU but are awaiting approval in the other market.
[2] Phase and planned filing dates refer to the lead indication in development.
[6] Resubmission to address FDA complete response letter