Research

We are a leader in the pharmaceutical industry in terms of research and development, including the level of our investment. The discovery and development of a new drug is a lengthy process, usually requiring approximately 10 to 15 years from initial research to market introduction, including approximately six to eight years from Phase I clinical trials to product launch. At each of these steps, there is a substantial risk that a compound will not meet the requirements to progress further, which may trigger the abandonment of the compound after substantial investments.

Our Research and Development teams work together to bring new and better medicines to market in the shortest possible time.

Novartis Institutes for BioMedical Research (NIBR)

The Novartis Institutes for BioMedical Research (NIBR) is the global pharmaceutical research organization of Novartis. With an ongoing focus on diseases for which medical needs remain unmet, scientists and physicians at NIBR are dedicated to ensuring that Novartis maintains its strong pipeline.

NIBR has sites in Basel, Switzerland; Shanghai, China; Cambridge, Massachusetts; and three other US locations.

Discovering new ways to improve and extend people’s lives

We currently have major research efforts focused on addressing some of the biggest health challenges the world is facing.

Cancer
Breast, lung, colorectal, kidney, pancreatic, bladder, melanoma, lymphoma and several types of leukemia

Diseases associated with aging
Macular degeneration, frailty, hearing loss, and tendon and muscle weakness

Neurological disorders
Autism, schizophrenia, bipolar disorder, and Alzheimer’s disease

Infectious diseases
Hepatitis B virus, respiratory syncytial virus, and human cytomegalovirus, clostridium difficile, staphylococcus aureus, and pseudomonas aeruginosa

Auto-immune disorders
Psoriasis, Sjogrens syndrome, and systemic lupus erythematosus

Ophthalmology
Diabetic retinopathy, retinitis pigmentosa, dry eye disease, uveitis, and glaucoma
Cardiovascular and metabolic diseases
Dyslipidemia, atherosclerosis and vascular diseases, type 2 diabetes, heart failure, cardiac arrhythmias and associated disorders

Respiratory diseases
Cystic fibrosis, idiopathic pulmonary fibrosis, pulmonary arterial hypertension, chronic obstructive pulmonary disease, and asthma

Diseases of the developing world
Malaria, dengue, chagas, and leishmaniasis

Pioneering innovative technologies and approaches for discovery
To accelerate the discovery of new medicines we are pioneering efforts to use innovative technologies and approaches to drug discovery. Some examples:

Chemical biology
Researchers have used the standard tools of biology and chemistry to develop many successful treatments, and we continue to employ them. But we recognize that these tools leave many drug targets – key proteins and nucleic acids known to play a role in disease – out of reach. We would like to hit these targets to fight disease, but they’ve dodged the conventional molecules in our arsenal. To address this challenge, we are blazing a new path: organizing our early discovery efforts around chemical biology. Chemical biology brings together experts from different fields – including biology, chemistry and computer science – to create new types of molecules and use them to probe biological systems. We have joined forces with researchers from the University of California, Berkeley, to form the Novartis-Berkeley Center for Proteomics and Chemistry Technologies, based in existing labs at Berkeley. The focus of this collaboration is on the development of new technologies for the discovery of next-generation therapeutics, pursuing the vast number of disease targets that have eluded traditional small-molecule compounds and are considered “undruggable.”

Genome editing
Genome editing, which enables scientists to change the DNA letters inside cells, has become a powerful research tool and technology for engineering cell and gene therapies. Though collaborations with leaders in this field, our scientists are working to harness this transformative technology to develop new therapies.

CRISPR, an acronym that stands for clustered regularly interspaced short palindromic repeats, is an approach that allows scientists to and precisely edit the genes of targeted cells. Think of CRISPR as a pair of molecular scissors that’s capable of snipping DNA. The tiny shears are combined with an RNA-based targeting molecule that scans the genome for specific sequences and makes a controlled cut at a single site. In a short period of time it has proven to be a powerful tool for creating very specific models of disease for use in drug discovery and has great potential for use as a therapeutic modality for treating disease at the genetic level by deleting, repairing or replacing the genes that cause disease. We currently have a collaboration with Intellia Therapeutics that is using CRISPR technology in conjunction with hematopoietic stem cells to explore treatments for sickle cell disease and thalassemia.

Homologous recombination is a biological mechanism used by cells to ensure highly precise DNA repair. Through a collaboration with biotech company Homology, our scientists are exploring the use of AAVs – I or adeno-associated viruses – that can be engineered to carry a specific genetic sequence and programmed to home to a target site in the genome. When they arrive, AAVs trigger homologous recombination, which overwrites a particular portion of a
gene or even replaces an entire gene. We are currently exploring the use of this technology to develop treatments or diseases of the eye and blood.

Our work with both technologies is in a preclinical stage.

**Immuno-oncology**

Tumors use molecular tricks to evade detection by the body’s natural defenses in our immune system. Our immuno-oncology research team is exploring new ways to harness this defense system to recognize and destroy tumors. The team is investigating three key steps in the immune response to cancer – education, activation and dissemination of immune cells to destroy tumors. They are seeking ways to improve immune cell activity at each step, and to combine these therapies with other treatments to offer the best outcomes for each patient.

**Digital**

Digital technologies have incredible potential to unlock the next chapter of explosive medical innovation. We are applying digital technologies such as machine learning in drug discovery and wearable sensors in early clinical development to better understand patient outcomes, capture digital endpoints, and to test how patients respond to our therapies.

**The exploratory phase of drug discovery**

All drug discovery efforts at Novartis focus on patients. Scientists determine which diseases will be the focus of research efforts based on two questions: do we have, or can we gain, significant understanding of the cause, or mechanism, underlying the disease? And does this disease represent a significant unmet medical need? If the answer to both questions is yes, then Novartis develops a research program aimed at better understanding the disease and finding an effective therapy. Early-discovery science determines disease cause at the molecular level, using our own discoveries as well as those from external collaborators. We look for clues in both patients’ experience of the disease and the compendium of historical medical and scientific knowledge, integrated with the growing knowledge of human biology, chemistry and genetics.

**Target discovery and drug design**

Typically, making a drug begins with identifying a protein associated with human disease. These proteins are known as “targets”. When it is confirmed that a target plays a role in a disease, an experiment known as a high-throughput screen is conducted to find a chemical compound or antibody that binds to or “hits” the target in a way that alters the disease. Once chemical compounds or antibodies are identified by their binding to a target, they are enhanced to improve their safety and effectiveness. The resulting chemical compound or antibody becomes a drug candidate.

**Preclinical safety and efficacy**

An initial profile of a drug candidate’s safety and effectiveness must be determined before it is tested in humans. In this phase, scientists use computer models and laboratory tests to assess the safety of a drug candidate. These tests determine how well a drug candidate is absorbed, where it goes within the body, how it is broken down or metabolized, and how quickly and in what manner it is eliminated from the system.
Proof of Concept

All drug candidates are taken to the clinic via "proof-of-concept" trials to enable rapid testing of the safety and efficacy of the drug while collecting basic information on pharmacokinetics and tolerability, and adhering to the guidance for early clinical testing set forth by health authorities. Following proof-of-concept, our Global Drug Development organization conducts confirmatory trials on the drug candidates.

Disclaimer

These materials contain forward-looking statements that can be identified by terminology such as "potential," "expected," "will," "planned," "pipeline," "outlook," or similar expressions, or by express or implied discussions regarding potential new products, potential new indications for existing products, or regarding potential future revenues from any such products; or regarding the potential outcome of the strategic review being undertaken to maximize shareholder value of the Alcon Division; or regarding the potential financial or other impact of the significant acquisitions and reorganizations of recent years; or regarding the potential impact of the share buyback plan; or regarding potential future sales or earnings of the Novartis Group or any of its divisions or potential shareholder returns; or by discussions of strategy, plans, expectations or intentions. You should not place undue reliance on these statements. Such forward looking statements are based on our current beliefs and expectations regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward looking statements. There can be no guarantee that any new products will be approved for sale in any market, or that any new indications will be approved for any existing products in any market, or that any approvals which are obtained will be obtained at any particular time, or that any such products will achieve any particular revenue levels. Nor can there be any guarantee that the strategic review being undertaken to maximize shareholder value of the Alcon Division will reach any particular results, or at any particular time, or that the result of the strategic review will in fact maximize shareholder value. Neither can there be any guarantee that Novartis will be able to realize any of the potential strategic benefits, synergies or opportunities as a result of the significant acquisitions and reorganizations of recent years. Neither can there be any guarantee that shareholders will achieve any particular level of shareholder returns. Nor can there be any guarantee that the Group, or any of its divisions, will be commercially successful in the future, or achieve any particular credit rating or financial results. In particular, our expectations could be affected by, among other things: global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures and requirements for increased pricing transparency; regulatory actions or delays or government regulation generally; the potential that the strategic benefits, synergies or opportunities expected from the significant acquisitions and reorganizations of recent years may not be realized or may take longer to realize than expected; the inherent uncertainties involved in predicting shareholder returns; the uncertainties inherent in the research and development of new healthcare products, including clinical trial results and additional analysis of existing clinical data; our ability to obtain or maintain proprietary intellectual property protection, including the ultimate extent of the impact on Novartis of the loss of patent protection and exclusivity on key products which commenced in prior years and will continue this year; safety, quality or manufacturing issues; uncertainties regarding actual or potential legal proceedings, including, among others, actual or potential product liability litigation, litigation and investigations regarding sales and marketing practices, intellectual property disputes and government investigations generally; uncertainties involved in the development or adoption of potentially transformational technologies and business models; general political and economic conditions, including uncertainties regarding the effects of ongoing instability in various parts of the world; uncertainties regarding future global exchange rates; uncertainties regarding future demand for our products; and uncertainties regarding potential significant breaches of data security or data privacy, or disruptions of our information technology systems; and other risks and factors referred to in Novartis AG’s current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in these materials as of this date and does not undertake any obligation to update any forward-looking statements as a result of new information, future events or otherwise.