Lernende Systeme

GERMANY'S PLATFORM FOR ARTIFICIAL INTELLIGENCE

Developing medicinal product with AI: From an idea to clinical licensing

White Paper

Budde, K. et al.
Working Group Health Care,
Medical Technology, Care

Executive Summary

The development of a drug is a complex, lengthy process: It takes an average of twelve years until market launch, at a total cost of around 2.8 billion US dollars. This process, from the initial idea to approval, can be streamlined with the use of artificial intelligence (AI) and thereby offers the opportunity to save years of laboratory work and costly investments: through systematic analyses in data processing, the identification of suitable drug candidates or the performance of clinical trials.

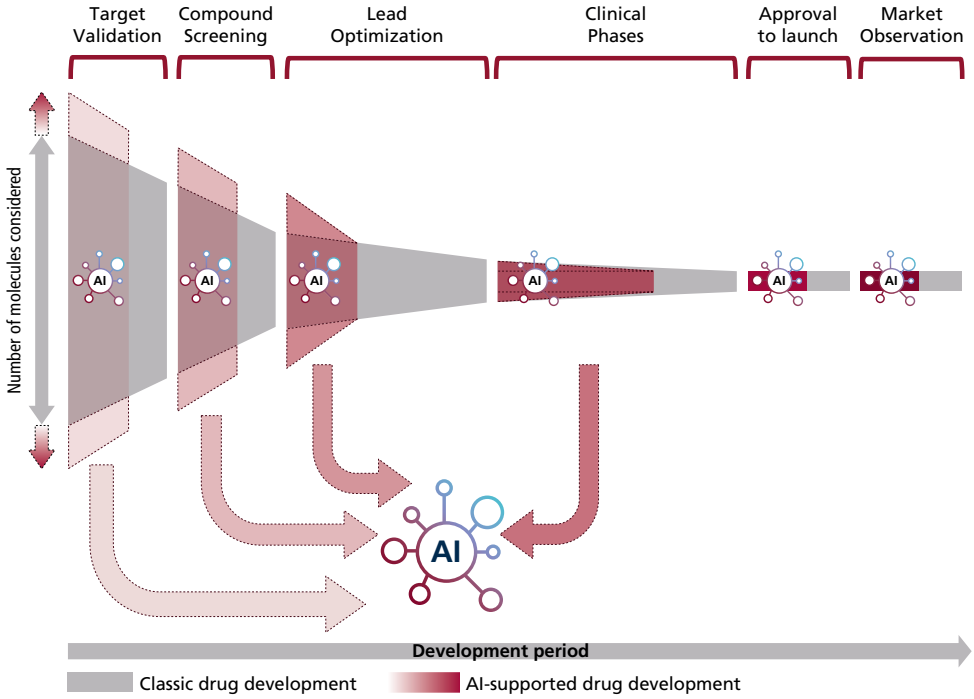
The cope for research provided by AI can be used productively in drug research to develop more effective and individualized drugs faster: for the company's own competitive advantage as well as for the benefit of patients. This requires not only a reliable data base on active ingredients and their side effects, but also sufficient high-quality patient data to be available for research. In addition to the willingness of research companies to share data, this in turn requires the active participation of the population in order to close gaps in the data base on human biology, especially for the development of personalized therapeutic approaches. This will require the establishment of a legally secure regulatory framework that guarantees the availability of health data for research purposes.

AI in drug development

AI technologies have enormous potential for companies in the pharmaceutical and biotech sector, particularly in terms of economic feasibility, by reducing cycle times and development costs for drugs, generating new hypotheses through expanded data use and changes in research culture. The use of AI enables companies to develop new business models and a digital ecosystem for the shared use of data. According to a survey, around 23 percent of pharmaceutical companies already use AI in drug development (Schulte, 2022).*

Drug discovery: AI technologies can be used in all phases of drug development; numerous applications already exist, particularly in the early phases, such as the identification of targets and leads.

Schematic representation of the advantages of using AI in drug development across all development phases: shorter development phases, more accessible molecules and utilization of all generated data to train the AI.



Source: Own compilation.

Right from the start, when **validating the drug target**, AI provides support by compiling and evaluating information from existing medical data bases, including literature data bases such as PubMed or gene and protein data bases, in order to identify targets that influence the course of the disease. This also enables the automated creation of knowledge graphs, which are particularly important for drug development in complex diseases.

* Schulte, A. (2022): Pharmaindustrie nutzt Künstliche Intelligenz zur Arzneimittelforschung. Online: https://www.handelsblatt.com/technik/medizin/neue-medikamente-pharmaindustrie-nutzt-kuenstliche-intelligenz-zur-arzneimittelforschung/28161478.html

AI also evaluates available data bases when selecting the **active ingredient structure** (lead identification), in particular on relevant substance properties such as absorption, diffusion, metabolism or protein interaction, in order to identify potential active ingredient candidates and predict their interaction with the target. This can also facilitate the repurposing of already approved active substances with the help of AI or completely new active substance structures can be created using generative AI – this can function in a similar way to generative language models such as ChatGPT, based on autoencoders. In the **phase of lead structure optimization**, in silico predictions (experiments simulated in the computer) provide further information on drug candidates: for example, on bioactivity and bioavailability, metabolism and excretion pathways in relation to human metabolism, including their physiological and pharmacological properties. In the **clinical phases**, AI improves the selection of participants in clinical studies, monitoring during the study and potentially enables new study designs: For example, filtering out suitable test subjects who match the development profile of the active ingredient before the start of a study, or creating clinical digital twins of participants based on available medical data as a virtual control arm of a study, as well as the selective recruitment of test subjects – especially for newer forms of therapy, such as gene therapy, or rarely occurring clinical pictures. AI tools also support the documentation for the **approval to launch** of the drug by significantly shortening this time-consuming consolidation process to prove the medical benefit and any side effects. In production, marketing and **market observation** (pharmacovigilance) of pharmaceuticals, AI also offers a wide range of potential applications for increasing efficiency and predicting trends: starting with the optimization of production design and process control, through predictive maintenance and trend monitoring of product quality based on customer complaints or deviations in the production process, to the creation of roadmaps for marketing. The integration of AI into drug development thus promises to significantly accelerate and improve the entire process as well as more effectively monitor the safety (pharmacovigilance) of drugs.

Personalized therapy approaches: Artificial intelligence can not only accelerate drug development, but its use can also promote personalized medicine across the board. This is because AI-supported data analysis enables the development of personalized therapies, for example for the treatment of cancer, which are tailored and adapted to the individual clinical picture of those affected, also with regard to concurrent findings. By means of retrospective studies, so-called real-world studies, based on real health data that are available in large quantities, the effect and/or side effects of an active substance can be evaluated in retrospect. These real-world studies are particularly useful for analyzing healthcare innovations for special population groups that are difficult to record in clinical studies, such as the very elderly, minors or cognitively impaired people.

Challenges

The use of AI in drug development has the potential to change the development and approval of medicinal products. However, in order to exploit the full potential and minimize possible risks, **appropriate regulations and guidelines for the validation of AI models and data sets must be developed for the use of AI**. The aim is to make health data for research purposes accessible to industry as part of a data ecosystem. It is therefore important to define clear standards for the evaluation of AI-based data and to ensure that AI-generated findings are transparent and verifiable. In particular, the problem of hallucination in medical language models must be addressed, which can already be partially compensated for by so-called evidence surfacing (extension of the AI statement with real citations from publications or references to data base entries as a basis for a validity check by humans). The efficient use of AI requires large amounts of **high-quality patient data**, for example from the electronic patient record (ePA) or from health insurance companies, in order to drive forward personalized medicine. The availability of data for AI in drug development can be improved by laws such as the draft regulation on the European Health Data Space (EHDS) and the Health Data Utilization Act (GDNG) in Germany. Finally, along with quality and availability, the **international connectivity of data sets for AI** in drug development is also crucial and requires **standardized data collection and interoperable data formats**, which includes international standards and clear rules for data use in addition to German and European standards.

Imprint
Editor: Plattform Lernende Systeme – Germany's Platform for Artificial Intelligence | Managing Office | c/o acatech | Karolinenplatz 4 | D-80333 Munich | kontakt@plattform-lernende-systeme.de | www.plattform-lernende-systeme.de | Follow us on X: @Lernende Systeme | LinkedIn: de.linkedin.com/company/plattform-lernende-systeme | Mastodon: social.bund.de/@LernendeSysteme | Status: March 2024 | Photo credit: gorodenkoff/iStock/Title | Translated using DeepL.com

This executive summary is based on the white paper *Arzneimittel mit KI entwickeln: Von der Idee bis zur Zulassung. Anwendungen, Potenziale und Herausforderungen*, Munich, 2024. The authors are members of the working group Health Care, Medical Technology, Care of Plattform Lernende Systeme. The original version of the publication is available at: *https://doi.org/10.48669/pls_2024-1*

SPONSORED BY THE

Federal Ministry of Education and Research

acatech
NATIONAL ACADEMY OF SCIENCE AND ENGINEERING