Assessing Pharmaceutical Innovation in Europe: Therapeutic Advances *versus* Unmet Public Health Needs

EPH20

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INTRODUCTION

Medicines are a fundamental component of healthcare and make a major contribution to health outcomes. The pharmaceutical industry develops and introduces a steady stream of new medicines each year, expanding the therapeutic options available for an increasing number of diseases [1]. In developed countries, the primary disease burden arises from noncommunicable conditions, with cardiovascular disease and cancer being the leading areas [2].

While available evidence suggests a positive correlation between therapeutic innovation and disease burden, there are concerns that certain disease areas may remain underrepresented [3, 4].

OBJECTIVES

The aim of this study is to assess the alignment between pharmaceutical innovation- measured through novel medicines[1] authorized by the European Commission (EC) between 2014 and 2024- and Western Europe's medical (unmet) needs, defined by the clinical burden of disease, highlighting existing gaps and opportunities.

1 - Defined as medicines which active substance is a new molecular entity or a new biological product, or novel fixed combination that had never been marketed in EU before;

METHOD

This retrospective observational study employs descriptive statistical analysis to characterize novel medicines authorized by the European Commission (EC) from 2014 to 2024. It further evaluates the relationship between innovation intensity - measured as the proportion of novel medicines relative to the total - and the burden of disease, with particular emphasis on conditions associated with the greatest disease burden and/or mortality.

A database was developed to characterize the medicines cohort according to molecule type, orphan designation, Marketing Authorization (MA) date, primary indication and therapeutic area, using publicly available data from EC and European Medicines Agency (EMA) databases and U.S. FDA annual reports.

The disease burden in Western Europe was assessed using disability-adjusted life years (DALYs) and mortality data obtained from the GBD Compare tool (IHME), expressed as a percentage of the total values for Western Europe in 2023.

To assess the alignment between pharmaceutical innovation and public health needs, the correlation between innovation intensity and disease burden were assessed using Pearson's coefficient.

RESULTS

A total of 481 novel drugs were approved by the EC, with 33% having orphan status. Although these medicines span for all 14 ATC level 1 categories, three areas dominate accounting for 56% of total. There has been a marked focus on oncology with a share of 28%, followed by rare diseases, with 17% (covering dozens of conditions), and 12% for Infectious diseases, mainly HIV and Hepatitis, with comparatively few approvals in areas such as new antibiotics (3%) and dementia.

Moreover, although small molecules still predominate (60%), their share has declined from 75% in 2014 to 53% in 2024. Biotechnological medicines - particularly monoclonal antibodies (which account for 64% of biotech products) -have increased, representing 32% of all approvals. Advanced therapy medicinal products (ATMPs) account for 5%, while oligonucleotides represent 2%. These trends reveal a shift toward medicines that are increasingly complex, specific in action, and highly targeted.

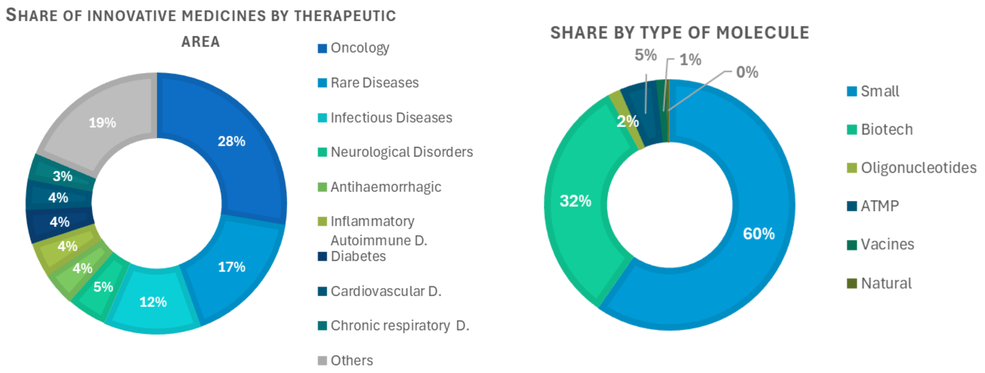

Figure 1 A - Share of novel medicines by therapeutic areas. Neurological Disorders (Migraine + Parkinson + Alzheimer + Multiple sclerosis + ALS); Infectious Diseases (ATC J01 to J05); Inflammatory Autoimmune Disease (Rheumatoid arthritis + Inflammatory Bowel Disease+ Lupus + Psoriasis) + Chronic Respiratory Disease (Asthma + Cystic Fibrosis (CF) + COPD); Figure 1 B - Share of the different type of molecules of novel medicines granted MA.

Eleven group of diseases (with different levels of desegregation and burden) were analyzed, as detailed in figure 2. Collectively, these areas accounted for 58% of total DALYs, 85% of total deaths, and 67% of the innovative medicines within the cohort.

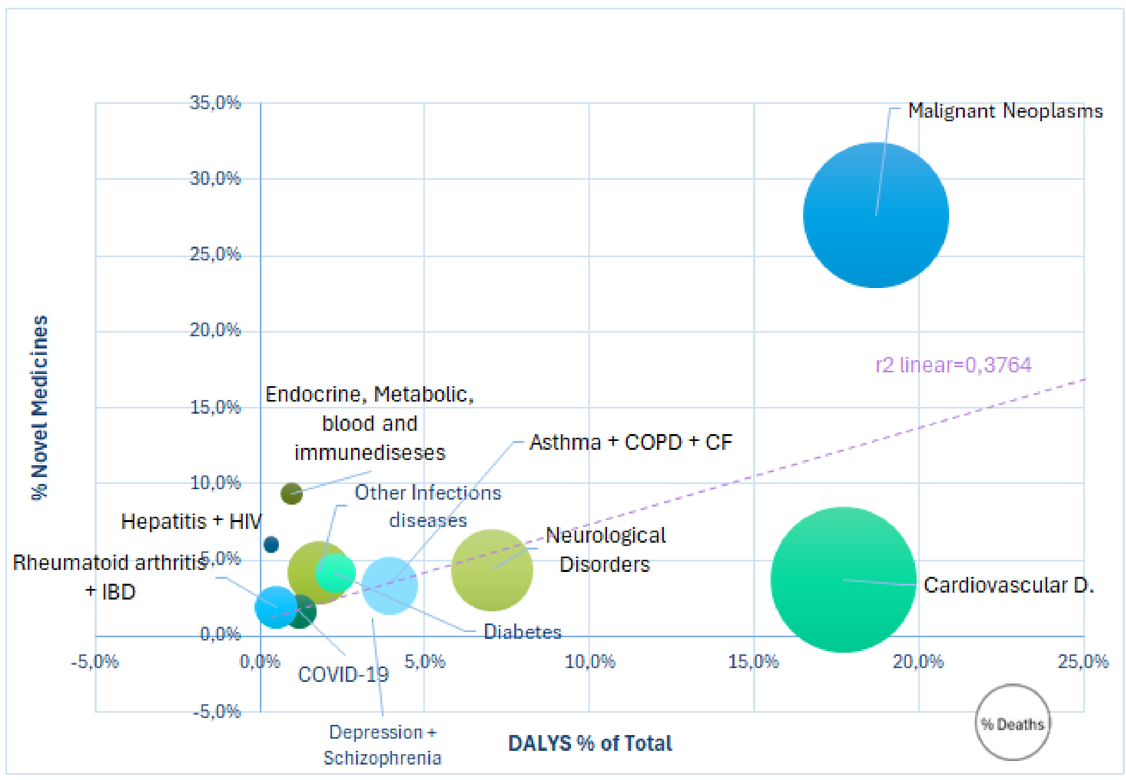

Figure 2 – Correlation between innovation intensity and DALYS for selected diseases. Endocrine, metabolic, blood, and autoimmune diseases (includes bleeding disorders, Psoriasis, Metabolic Diseases and Thyroid Diseases); Other Infections diseases (Infections diseases except Covid19, HIV and Hepatitis)

As shown in Figure 2, the relationship between disease burden and innovation intensity varied across therapeutic areas. A moderate positive correlation was observed between the two variables (Pearson's r = 0.6105), which was statistically significant at the 5% level (p = 0.0461). However, when the two outliers - malignant neoplasms and cardiovascular diseases - were excluded, the correlation dissipated (Pearson's r = –0.212).

Having similar disease burdens, Neoplasms demonstrated a disproportionately high innovation intensity, in contrast to cardiovascular diseases. Moreover, neurological disorders, which account for 7% of total DALYs, received less innovation than endocrine, metabolic, blood, and autoimmune diseases, despite their comparatively lower disease burden, suggesting a potential misalignment of innovation priorities. Additionally, a notable gap was observed in the development of new antibiotics, despite the growing challenge of antimicrobial resistance.

CONCLUSIONS

New medicines play a crucial role in advancing public health; however, gaps persist between innovation and actual health needs, particularly in areas with high disease burden and limited therapeutic options.

Therapeutic innovation is influenced by multiple factors affecting return on investment, such as market forces, scientific challenges, etc., of which disease burden is only one, and our analysis clearly indicates that factors beyond disease burden drive the development of new therapies.

Policy implications may emerge from these findings. This imbalance should be considered when setting research and funding priorities, highlighting the need for targeted incentives - drawing on past approaches used to address unmet needs, such as with orphan drugs - to stimulate investment in high-burden diseases that currently lack effective therapeutic options.

REFERENCES

[1] F. R. Lichtenberg, "THE IMPACT OF NEW DRUG LAUNCHES ON LIFE-YEARS LOST IN 2015 FROM 19 TYPES OF CANCER IN 36 COUNTRIES," J Demogr Economics, vol. 84, no. 3, pp. 309–354, Sep. 2018, doi: 10.1017/dem.2018.11.

[2] A. J. Ferrari et al., "Global incidence, prevalence, years lived with disability (YLDs), disability-adjusted life-years (DALYs), and healthy life expectancy (HALE) for 371 diseases and injuries in 204 countries and territories and 811 subnational locations, 1990–2021: a systematic analysis for the Global Burden of Disease Study 2021," The Lancet, vol. 403, no. 10440, pp. 2133–2161, May 2024, doi: 10.1016/S0140-6736(24)00757-8.

[3] F. Catalá-López, A. García-Altés, E. Álvarez-Martín, R. Gènova-Maleras, and C. Morant-Ginestar, "Does the development of new medicinal products in the European Union address global and regional health concerns?," Popul Health Metr, vol. 8, Dec. 2010, doi: 10.1186/1478-7954-8-34.

[4] O. I. Martino, D. J. Ward, C. Packer, S. Simpson, and A. Stevens, "Innovation and the burden of disease: Retrospective observational study of new and emerging health technologies reported by the EuroScan network from 2000 to 2009," Value in Health, vol. 15, no. 2, pp. 376–380, Mar. 2012, doi: 10.1016/j.jval.2011.11.034.

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