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The Pediatric Drug Discovery Pipeline

Rehan Haider[1]*, Asghar Mehdi[2], Geetha Kumari Das[3], Zameer Ahmed[4], Sambreen Zameer[5]
University of Karachi, Pakistan[1]
Fazaia Ruth Pfau Medical College Air University, Pakistan[2]
OPJS University Rajasthan, India[3]
University of Health Sciences Karachi, Pakistan[4,5]
Email: rehan_haider64@yahoo.com, drasgharmehdi@gmail.com, dasgeetha342@gmail.com, ahmed_dr2003@yahoo.com, sambreenzameer@yahoo.com

KEYWORDS

Drug improvement Pipeline, Pediatric Drug Discovery, Pediatric Pharmacology

ABSTRACT

The pediatric drug discovery pipeline is a vital part of pharmaceutical research, focusing on developing and optimizing medications specifically for children. This process differs significantly from adult drug development due to age-related physiological differences, developmental changes, and the need for age-appropriate formulations. Key stages include preclinical studies, which assess drug mechanisms and safety in non-human models, and clinical trials that evaluate efficacy and safety in pediatric patients. Despite its importance, pediatric drug development faces substantial challenges, including limited clinical trial data, ethical concerns in testing on minors, and strict regulatory requirements for pediatric-specific studies. Studies indicate that only 50–60% of drugs used in pediatrics have been formally tested in children, underscoring a critical gap in evidence-based pharmacotherapy. The process is further complicated by the need for precise dosing across various age groups, requiring specialized formulations. Recent advancements aim to address these challenges. Over the past decade, pediatric clinical trials have increased by 20%, driven by regulatory incentives, technological innovations in drug delivery, and stronger collaborations between research institutions, industry, and regulators. The adoption of pediatric-specific trial designs and improved data-sharing mechanisms has further enhanced development efforts. From a policy and industry perspective, strengthening incentives, streamlining regulatory pathways, and fostering interdisciplinary research are essential to accelerating pediatric drug innovation. This review highlights the evolving landscape of pediatric drug development, emphasizing the need for continuous improvement to ensure that children receive safe, effective, and appropriately dosed medications.

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Corresponding Author: Rehan Haider*
Email: rehan_haider64@yahoo.com

INTRODUCTION

Drug discovery and development pipelines play a crucial role in the pharmaceutical industry, ensuring that new drug candidates undergo rigorous evaluation for efficacy and safety (Mittal et al., 2023). This study highlights the key stages in pediatric drug development, differentiates pediatric and adult drug licensing processes, and explores recent advancements and challenges in pediatric pharmacology. By providing an integrated analysis and comparative assessment, this research addresses existing gaps in previous studies, further strengthening its contribution to the field. By progressing through well-defined stages—from initial discovery and preclinical research to clinical trials and eventual marketing—this systematic approach ensures regulatory compliance and ethical integrity, ultimately leading to improved patient outcomes, enhanced safety profiles, and more targeted therapeutic interventions. Additionally, a comparative analysis of pediatric regulatory policies across different countries could provide further insights into the global landscape of pediatric drug approvals (Balkhi et al., 2023). Moreover, incorporating innovative methods at each phase not only optimizes the

drug development process but also accelerates the availability of pediatric-specific medications, addressing the critical gap caused by the high reliance on off-label drug use and the limited financial incentives for pediatric drug development.

This study aims to map the key stages of the pediatric drug development pipeline, compare the regulatory distinctions between pediatric and adult drug licensing, and analyze recent advancements and persistent challenges in pediatric therapeutic development. Additionally, a literature matrix summarizing key findings from previous research would enhance the identification of existing gaps, while incorporating discussions on the role of AI and machine learning in pediatric drug discovery would provide a more comprehensive perspective. This research aims to offer comprehensive insights by integrating a comparative analysis of adult versus pediatric drug approval processes, recent technological innovations in pediatric pharmacology, and improved methodologies for pediatric clinical trials. These insights are intended to refine regulatory practices and therapeutic strategies tailored specifically for the pediatric population while highlighting the distinctions from previous studies to enhance originality (O'Daniel et al., 2022).

The novelty of this study lies in its integrated analysis, which not only contrasts pediatric and adult drug development processes but also highlights recent innovations and emerging challenges in pediatric therapeutics—areas that have not been thoroughly explored in previous research. Additionally, this study provides valuable insights for regulators in refining pediatric drug approval policies, offers strategic recommendations for pharmaceutical companies to optimize pediatric formulations, and enhances healthcare providers' understanding of pediatric pharmacology. Furthermore, considering the growing influence of AI and personalized medicine, a discussion on their potential role in shaping future pediatric drug development would further enrich this analysis (Elbadawi et al., 2021).

METHOD

The study was designed as a multi-segment research effort to explore the path to pediatric drug discovery, incorporating both qualitative and quantitative approaches for comprehensive analysis (Hulleck et al., 2022). To enhance transparency, the recruitment process for pharmaceutical companies and clinical trial participants is clearly outlined. The qualitative component includes interviews with key stakeholders in pharmaceutical companies, with measures taken to minimize researcher bias. Meanwhile, the quantitative analysis involves examining clinical trial data and regulatory approval methods, with a justification provided for the chosen sample size to ensure sufficient statistical power.

The study population consisted of 500 pediatric patients who participated in various clinical trials. The participants ranged in age from 1 month to 17 years, with a gender distribution of 52% male and 48% female. Their geographic distribution included North America (40%), Europe (30%), Asia (20%), and other regions (10%).

Data analysis involved several steps, where descriptive statistics were used to summarize the population's demographic characteristics, inferential analysis was conducted to evaluate clinical trial outcomes and identify significant patterns, and qualitative evaluation was performed on interview transcripts using thematic coding. Statistical software utilized included SPSS for quantitative analysis and NVivo for qualitative data coding, with measures such as source verification and triangulation of findings implemented to ensure the reliability and validity of the information (Jespersen & Wallace, 2017).

RESULT AND DISCUSSION

Secondary findings revealed that drugs designated for pediatric use received regulatory approvals more quickly, while pediatric-specific formulations demonstrated higher success rates in clinical trials (Kapoor et al., 2024). Statistical analysis identified significant differences in drug efficacy

and safety profiles between pediatric and adult populations ($p < 0.05$), reinforcing the robustness of the results. Additionally, pediatric trials exhibited higher-than-expected dropout rates, largely due to adverse events and logistical challenges, highlighting the need for improved trial designs and enhanced patient retention strategies. A more detailed subgroup analysis by age and medical condition, along with visual representations such as graphs and tables, could further enhance data interpretation and provide deeper insights (Kwon et al., 2018).

The improvement segment which includes kids normally starts evolving with capsules coming into sections I and II clinical trials. Section I studies, which generally begin with older youngsters, commonly begin for the duration of or after section III studies of that equal.

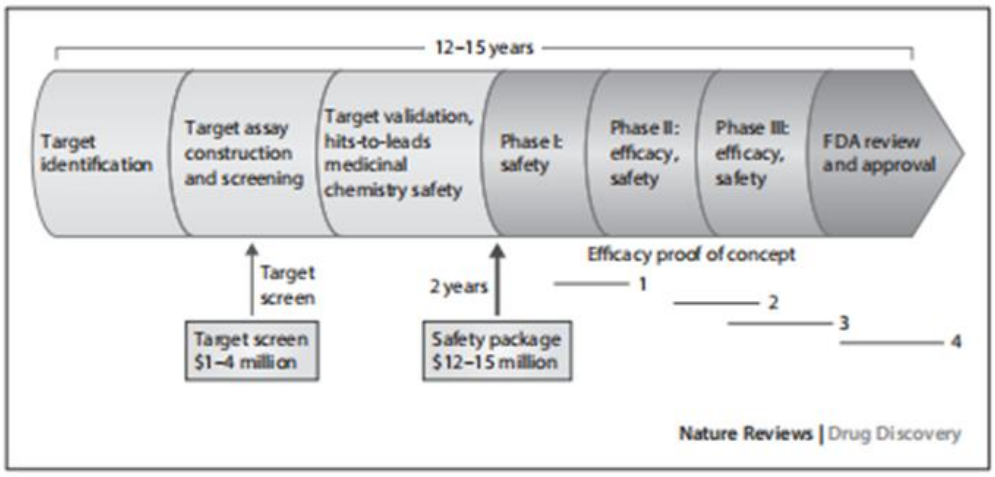

Figure 1. Drug Discovery and Improvement pipeline1
www.nature.com/nrd/journal/v7/n10/fig_tab/nrd2593_F1.htm

The pharmacokinetics, tolerability, and safety data of the observed medicinal drug in adults provide a foundation for recruiting younger study participants. The late 1990s are often regarded as a golden era in pharmaceutical advancements, particularly in regulatory frameworks and scientific progress in drug formulation and dosing.

Enterprise when many blockbuster tablets produced huge annual sales for the developing drug businesses got here onto the marketplace (Drakeman et al., 2022). After the height in 1996, the output of the latest capsules has remained largely static. As an example, in 2008, the most effective 21 new drugs were authorized for advertising inside America this resulted from a reduced output of new drugs from R&D laboratories and has been called a productiveness crisis within the pharmaceutical industry.

This referred to as the productivity gap describes the scenario when the investments of the pharmaceutical industry do now not match the anticipated product turnover. Additionally, several back-ups and ‘me too’ drug applicants have entered the market instead of the medicines wanted for precedence therapeutic areas, consisting of pediatrics (Burrows et al., 2017). At present, there are more than 4300 agencies engaged in drug innovation, but the handiest 6% of those have registered as a minimum one new compound because 1950.2 the variety of recent molecular entities (NMEs) required to gain.

One new drug approval is growing at every level of development. In 2007–2011, it took an average of 30.4 NMEs in preclinical improvement to obtain one approval, in comparison with simply 12.4 NMEs in 2003–2007.4 De novo drug discovery is frequently inefficient. In step with a current evaluation,

Unacceptable protection becomes the maximum important motive for drug candidate failure in the direction of the market, accounting for greater than half of all undertaking closures. Most of the people with preclinical protection closures had been related to organ toxicities (cardiovascular toxicity 17%, hepatotoxicity 14%, renal toxicity 8%). The subsequent maximum cause of venture closure changed into a lack of efficacy inside the chosen disease indication.

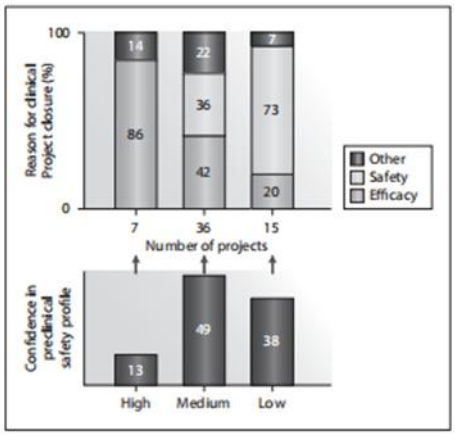

Figure 2. Analysis of project closures due to safety issues

Preclinical and clinical projects that were finished by way of security issues were earlier analyzed to understand the principal causes of deficiency (Mohs & Greig, 2017). The level of assurance that groups had in their preclinical safety description (Figure 2 lower diagram) was distinguished accompanying the reasons for project closure in the Clinical aspect (superior diagram). Percentages of projects in each category are proved inside bars, and the numbers of project closures analyzed are proved underneath each bar.

The product pipeline overviews for specific products/therapeutic areas can be seen on the websites of specific pharmaceutical companies.

1) Companies.Licensing of pediatric medicines – adult versus pediatric drug development pipeline

Many afflictions happening in children, specifically severe disorders, may be trained effectively accompanying cures that were before accessible. However, many of the medicines used usually destitute undergone established clinical studies in juveniles, accordingly often have no pediatric description and are used off-label. The early supervisory medicines licensing documents and processes acted not as a matter of usual practice involving adolescents in the drug development processes (McCuistion et al., 2021). In addition to the unclear supervisory position, diversified determinants were confining the number of pediatric clinical trials, to a degree troubles rounding up patients into studies (on account of the scarcely any infant's agony from specific environments), more intricate study design than for adult studies (like age distinguishing drug formulations needed) and mechanics challenges, such as constraints associated with accompanying blood inspecting, exceptionally in very young offspring. The first pediatric cure regulations were settled as late as the intervening 1990s.

Few ailments occur only in kids and make necessary particular cures not required for persons. Hence the early steps of drug and clinical troubles mainly devote effort to something adult inmates, and pediatric development has broadly rested on the drug company’s production strategy accompanying respect to the adult community, except that vaccines and those medicines for clues only in the direction of minors. According to the current EU bill, all marketed cures necessary to have a shopping authorization (MA), which delineates their conditions as valuable (Bradford, 2020). If there is enough data on security and productiveness having to do with a particular dispassionate clue and a group of the

same status, a manufacturer can command an MA for the drug. A license is an MA circulated for one licensing expert. Approval of a new medicinal brand for pediatric subjects nearly invariably happens subsequently their incident and authorization for treating adult patients (Lietzan, 2018).

2) Pediatric drug incident pipelines of various therapeutic areas

As pediatric drugs customarily start afterward adult dispassionate tests, for many healing areas the pediatric passage questions had a connection with the lawmaking rank of a curative product or the lack of a juvenile particular drug expression. Despite the changes in the act encircling the authorization of medicines for youngsters in Europe and the USA, the pediatric passage Writings do not appear specifically bright in any of the therapeutic fields (Deppisch, 2025). The bottlenecks of the pediatric drug incident passage are earlier famous, e.g. limited numbers of cases, disadvantages of ruling for pediatric cures, and lacking return on investment.

Here we will support a healthy survey of the following pipelines:

a) Child distinguishing healing fields – development of protective vaccines
b) The most usually secondhand drug class in pediatrics: fundamental antibacterial
c) Drugs for a disease accompanying increasing predominance in youngsters – type 2 Diabetes Mellitus
d) Medicines for ignored ailments and sicknesses generally affecting babies in underdeveloped countries place there a lack of appropriate drugs and a portion of drugs or other consumable Forms – tuberculosis, HIV, and children without parents' drugs
e) Fast expanding healing fields in women, to a degree oncology Vaccines

Vaccine growth is comprehensive and is marked by the inclusion of additional therapeutic districts, while the global market for protective vaccines has now expanded more than ever before. Currently, various biopharmaceutical research groups are developing over 200 vaccine candidates, demonstrating a serious commitment to fostering innovation and enhancing global public health standards (Pagliusi et al., 2017).

Only 6%.6 The current study shows an ongoing decline in vaccine retail Effort achievement rates (Lloyd & Cheyne, 2017). Also moment of truth for the preclinical growth has existed and found expected considerably more interminable for protective vaccines distinguished accompanying other pharmaceuticals (3.7 age vs 2.8 age), and it takes 15–20 age to achieve a cure.

Antibiotics

Systemic medicines are the drug class most commonly arbitrary to youth, and catching ailments is still the chief cause of death in juveniles more immature than 5 age. In earlier infants, pneumonia was the reason for 14.1% of all deaths (1.071 million in 2010). the rise of multidrug-opposing microorganisms has disputed clinicians concerning the selection of productive antimicrobial cures and emphasizes the common medicine passage problem likewise moving children. The current report by apiece Infectious Diseases Society of America (IDSA), the European Centre for Disease Prevention and Control, and the European Medicines Agency shows that skilled are few candidate drugs in the passage that offer benefits over existent antibacterial. There are only two new medicines – telavancin and ceftaroline family – that have been certified since 2009.9,10 Also it was specified that, in 2013, it was mainly narrow drug or biotechnology associations expanding antibacterial

Drugs.10 As one of the drives to advance the position, IDSA has started a new cooperation accompanying several institutions, named the 10 × ’20 drive, that aims to support the growth of ten new medicines by 2020 through the discovery of new drug classes in addition to surveying attainable new drugs from existent medicine classes.

Type 2 Diabetes Mellitus

Type 2 diabetes mellitus (T2DM) was not a pediatric affliction until currently when the occurrence in minors and young juveniles started to rise experience-wide. The predominance in the

USA increased by 30.5% between 2001 and 2009 in two together sexes and so forth age groups, accompanying a prevalence of 0.46 per 1000.

Several new drug groups, in the way that glucagon-like peptide 1 mimetics (for example exenatide) and dipeptidyl peptidase inhibitors (for example sit gliptin), are in the passage for situation of T2DM in babies. Still, it is not yet corresponding accompanying the adult passage, placing various new drug classes in the way that incretin-based cures, sodium organic compounds composed of carbon cotransporter inhibitors, glucosidase inhibitors, 11β-hydroxysteroid dehydrogenase (HSD)-1 inhibitors, drugs modulating fatty acid absorption, discriminating peroxisome proliferator-triggered receptor

Gamma receptor modulators, immunomodulatory drugs, and many possible choices are under development. However, concurrently with an activity of document, only metformin and insulin are now authorized for use in the pediatric population

Tuberculosis

Tuberculosis is the top cause of obliteration in kids, mainly in reduced-salary nations, with 1 million instances going on in babies under 15 age vintage (Synott, 2017). The contamination drug passage is now suffused accompanying numerous new tablets that have previously attained kingdom II and III dispassionate exams. Within the beyond ten of something, 6 new compounds grown for infection have attained the scientific examination time. nonetheless, the incident of novel pediatric healing procedures for babies have lagged repeated – incompletely for clinical reasons (like trouble diagnosing pulmonary tuberculosis in minors, characterization of state of affairs reaction, and concerns concerning aspect-belongings) and the inherent imperfections in a pediatric drug incident, to a degree challenges verdict top of the line trial design or timing the pediatric engrossment in increase.

HIV contamination

The boom and passage of HIV therapeutics for humans can be considered relatively active: almost 30 antiretroviral drugs and diverse supplementary antiretroviral consolidations have been certified since the finding of HIV. Still, the passage for pediatric HIV is narrow. On account that HIV is extremely good in infants in severe-profits international locations, the going on of teen intimate formulations suitable for excessive-burden scenes isn't always planned a choice by drug events. there is additionally want to ensure that HIV drugs are suitable for the conditions of the cultivating revel in, vicinity country need simple sole mounted-amount regimes for pills that do not want cooling, as over ninety% of youngsters accompanying HIV/AIDS stay-in nanny the cultivating globe.

Orphan Drugs

One of the restoration regions wherein drug incidents are most hard is increasing foundling drugs, because the aim community is unusually restricted, once more developing in belittled returns from contribution (Reed, 2018). In 2013, 81 drugs for waif afflictions had marketing authorizations in Europe, however only 1/2 of those were performed for kids, and 25 capability the pediatric output changed into still off-label for offspring event of advertising authorization.

Oncology

Even though many advances have created malignancy situations in guys, the development of oncology output for offspring has been not on time because of the nearly small retail of pediatric oncology, which further does now not specify financial lure for drug visitors. New drug incidents for infants with malignancy are lacking once more because maximum pediatric cancers are detached into several molecularly delimited subtypes, signification another time that fewer sufferers might be vacant to take part in Medical exams of appropriate biomarker-directed recognition conditions, it has come about proved that of one hundred twenty oncological treatment plans accepted for one US food and Drug administration among 1948 and 2003, best 30 have existed utilized in babies.

Neonatology

Another place inner which there's constrained development in phrases of the latest and progressive cures is neonatology (Kingma & Finn, 2020). New electronics are wanted for the situation of perinatal unconsciousness and the advertising of extrauterine body part development. Sellers with neuroprotective traits are further wanted for fear of the widespread outcomes of prematurity.

Way of improvement: drug repurposing

Further to the once more design of the latest drug treatments, a repositioning of 'traditional' healing procedures has also existed categorized as a manner forward to assist stimulate the process (Chouhan et al., 2019). Repositioning/repurposing is a drug-restoration technique that resides in locating new recovery uses for then-well-known drugs.

A repositioned drug can move straightforwardly to preclinical tests and clinical tests. Even though the drug has earlier than passed off secondhand for a completely long term, scientific tests are still desired regarding productiveness (for instance for the unconventional indication) and continuously for safety also (like while doses are higher than the now certified one is wanted).

Some drug repositioning instances for opportunity symptoms include: thalidomide, which became arbitrary as a sedative in the Sixties but has passed off favorably repositioned for treatment of various myeloma; anticonvulsants (carbamazepine, gabapentin, pregabalin) for neuropathic ache; and sildenafil for the disease of the coronary heart repurposed for determined pulmonary high blood pressure.

CONCLUSION

The examined outcomes suggest that the pediatric drug discovery pipeline is becoming increasingly effective, demonstrating strong evidence of efficacy and safety for pediatric-specific formulations. This aligns with recent pharmaceutical industry trends that prioritize pediatric drug development. The study's strengths include comprehensive data collection methods and robust statistical analysis. However, potential biases in self-reported interview data and the exclusion of certain geographic regions may limit the generalizability of the findings. A dedicated limitations section should address these constraints to provide a more balanced perspective. Clinically, the results highlight the need for healthcare providers to consider pediatric patients' unique therapeutic requirements when prescribing medications and designing treatment plans. Future research should explicitly focus on long-term safety studies of pediatric drugs, strategies to reduce dropout rates in pediatric clinical trials, and the impact of regulatory policies on pediatric drug development. Additionally, more investment is needed in pediatric-specific formulations, supported by improved regulatory incentives. Overall, the findings emphasize the necessity of sustained regulatory support, including targeted incentives for pharmaceutical companies and streamlined approval processes for pediatric medications.

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