Precision Health: Emerging innovations for children and adolescents

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Note: The findings, interpretations and conclusions expressed in this science-policy brief are those of the researchers and authors, and do not necessarily reflect UNICEF policies or approaches.

Abstract

This science-policy brief explores precision health innovations with potential impact for children. Ensuring healthy lives and promoting well-being at all ages is a cornerstone of sustainable development. Advances in life sciences, medicine, and technology have offered opportunities to find new ways of keeping people healthy. The brief outlines the unique characteristics of precision health technologies, explores the potential of precision diagnostic and therapeutic technologies to address high-priority health problems affecting children, and proposes actions and policy directives that would advance precision health for health equity.

Ensuring healthy lives and promoting well-being at all ages is a cornerstone of sustainable development. Although progress has been made in improving the health of millions of people and reducing some of the common killers associated with child and maternal mortality, we need to do more to discover causes and cures for a range of persistent diseases and health challenges that impose a heavy burden on children and their families, particularly in low- and middle-income countries (LMICs). The emerging field of precision health, shaped by research and development at the interface of biological sciences, synthetic biology, bioengineering, biomaterials science, chemistry, and nanoscience, offers new ways to keep people healthy.[i]

What is precision health?

The current model of healthcare is designed for the average patient and based on 'signs-and-symptoms'. The one-size-fits-all treatments can be successful for some patients but not for others, and some even experience negative side effects. Precision health, on the other hand, considers differences in people's genes, environments and lifestyles and formulates prevention strategies and treatments based on their unique backgrounds and conditions.[ii] Precision medicine uses genomics to inform drug discovery and allows the tailoring of medical treatment of disease and disorder. Big data technologies and increasing AI are key to achieving these goals. These technologies generate a volume and variety of data and sophisticated analytics not previously possible for disease surveillance, risk prediction and treatment optimization.

The promise of precision health

The uniqueness of precision health can be characterized by the 4Ps:

- Predictive - identifying and understanding intrinsic and extrinsic risk factors.
- Preventive - addressing risk and protective health factors prior to the development of disease and disorder.
- Personalized - optimizing treatments based on individual's need.
- Participatory - informing and involving the patient in decision-making.

Precision health approaches can strengthen health systems in two ways. Firstly, they surface insights into the biological, environmental, and behavioural influences of diseases and why certain individual and population groups are susceptible to a particular disease and their response to a specific treatment. Secondly, precision medicine introduces innovative medical products, vaccines and technologies that can improve health outcomes.

Precision health technologies and innovations

An accurate diagnosis is the first step for patients to receive effective treatment. Next generation diagnostics technologies, such as liquid biopsy, microfluidics (lab on a chip), 4D sonography, wearable and implantable biosensors, and soft nano robotic systems have allowed for frequent monitoring of important disease-specific biomarkers, more precise diagnosis, and point-of-care testing.

Next generation sequencing technologies have revolutionized genomic research, with major implications for clinical practice. These technologies provide insights into the biological context of disease mechanisms and can be applied to sequence whole genomes and target regions; discover novel RNA variants; analyze epigenetic factors and identify novel pathogens.[iii]

Genetic therapies are a group of techniques developed to correct defective genes and to explore the use of

genes to cure or treat certain diseases.[iv] Gene therapies treat or cure genetic conditions by removing a stretch of DNA that causes a disease; turning off a gene to prevent it from making a harmful protein; turning on a gene or instructing a cell to make more of a needed protein, and repairing the mutated gene within the cell.[v] For example, CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats) technology opens the door to editing genes that cause many diseases including cancers such as leukaemia and lymphoma, blood disorders, genetic disorders, diabetes, chromic infection, inflammatory disease, cardiovascular disease, HIV/AIDS, and muscular dystrophy.

The growing understanding of the crucial roles of RNA (Ribose nucleic acid) and the success of mRNA (messenger RNA) vaccines against COVID-19 have renewed clinical interest in the development of RNA-based therapeutic technologies. A new generation of RNA diagnostics and therapeutics draws on the ability of RNA to stimulate immune responses and deliver therapeutic proteins for potentially a wide spectrum of microbial pathogens and cancers and improve our understanding the root causes of neurodegenerative diseases.[vi]

Applications for children and adolescents

The potential applications of precision health technologies for children and adolescents include communicable diseases such as malaria, diarrhoeal diseases, tuberculosis, pneumonia, and HIV/AIDS; non-communicable diseases such as asthma, childhood cancer, and rare diseases such as sickle cell anaemia. Many of these diseases are major health concerns for children.

Table.

Health challenge	Potential of precision health technologies
Malaria • A child dies of malaria nearly every minute.[vii]	• Help identify high risk phenotypes and progression pathways in severe malaria, especially children with cerebral malaria and respiratory distress who are at the highest risk of death. • Advance the development of transmission-blocking malaria vaccines.[viii]
Diarrhoeal diseases • About 9 per cent of all deaths among children under age 5 are caused by diarrhoeal diseases.[ix]	• Help identify pathogens and detect the emergence of new variants. • Support the development of potential vaccines that promote immunity.[x]
Tuberculosis (TB) • Over 1 million children fall ill with TB every year.[xi] • Drug resistant strains of *Mycobacterium tuberculosis* are slowing progress to eliminate TB by 2030.[xii]	• Adopt precision medicine technology for next generation sequencing of the *mycobacterium tuberculosis* genome to the development of next generation of TB vaccines.[xiii]
Pneumonia • A child dies of pneumonia every 43 seconds.[xiv]	• Develop novel biomarkers for viral pneumonia using meta-genomics to differentiate viral from bacterial pneumonia.[xv] • Identify new genomic regions linked to the risk for the disease, thus paving the way for potential new treatments.[xvi]
HIV/AIDS • About 2.8 million children and adolescents are living with HIV/AIDS.[xvii] • Approximately 13.9 million children under the age of 18 had lost one or both parents to AIDS-related causes.[xviii]	• Generate insights on human and retroviral genomes interactions and immune non-response to antiretroviral therapy.[xix] The increased use of HIV/AIDS medicines has led to drug resistance, especially in children under 18 months of age.[xx] • Contribute to HIV/AIDS vaccine discovery. In 2022, Moderna and IAVI started Phase 1 clinical trial of a HIV/AIDS vaccine using Moderna's mRNA technology.[xxi]
Asthma	• Use omics sciences to identify genetic variants and assess asthma

• Asthma is the most common chronic disease among children.[xxii]	phenotypes.[xxiii] • Improve understanding of how genetic factors, biomarkers, environmental exposures interact during a disease process and during treatment.
Childhood cancer • 400,000 children aged 0-19 years develop cancer every year.[xxiv] • The heterogeneity of malignancies, the limited success of targeted drugs, and the absence of reliable biomarkers make diagnosis and therapeutic interventions difficult.[xxv]	• Apply next-generation RNA sequencing technology to improve new molecular understanding of childhood cancers.[xxvi] • Develop of potential cancer vaccines, including RNA vaccines, and other emerging therapeutic approaches such as epigenetic approaches, immunotherapeutic approaches and target fusion gene products.[xxvii]
Rare diseases • 50-75 per cent of rare diseases affect children.[xxviii] • Only about 5 per cent of rare diseases have approved treatments.[xxix]	• Apply whole-exome sequencing or whole-genome sequencing technology for precise genetic diagnosis.[xxx] • Offer the world's first commercial CRISPR treatment for sickle cell disease, which was approved by UK and US health authorities in late 2023.

Subheading

The potential benefits of precision health are not distributed evenly, either within or between populations. If precision health is to fulfil its potential, it needs to address the risk of exacerbating health inequalities and evolve to a paradigm that leaves no one behind. Below are four areas for anticipatory policymaking.

1. Science diplomacy - partnerships for development. North-South and South-South cooperation and partnerships are critical. Countries with significant capacity in precision health research should be encouraged or incentivized to collaborate with under-and unrepresented countries. These must be mutual learning experiences where equitable arrangements are made to ensure fair benefit sharing, respectful data, and sample handling. Genomics research has focused mainly on populations of European descent, resulting in significant knowledge gap about populations with substantial genetic diversity that reflect their historical context and evolutionary adaptations. As a result, many underrepresented people miss out on the potential benefits of precision health and researchers miss out on potential insights that could lead to better solutions.

2. Business models for health equity. The tension between affordability and profitability underpins the challenge of health equity. Precision health innovations are expensive endeavours involving lengthy periods of research, translating research into potential drugs and financing for clinical trials, all without guarantee of success. Currently, large biopharmaceutical corporations dominate the R&D space and the market. Financial and non-financial incentives must be designed to sustain the 15+ year journey from scientific discovery to service delivery, thereby allowing other actors to enter and influence the market. Business models must also be redesigned to improve health equity and achieve sustainable health outcomes.

3. Life course approaches. The integration of life course approaches to precision health research and practice would help reduce the risk of health disparities. Life course approaches emphasize how socially patterned exposures to risk factors during sensitive life stages shift health trajectories and how social identity and position disproportionately allocate risk factors and resources and alter health trajectories.[xxxi] These approaches stress the importance of acting early in the life course and place children and adolescents at the centre of precision health innovations.

4. Data governance. Using big data in precision health has risks and more work needs to be done both enumerating and evaluating these risks. What is needed at minimum are enforceable data governance frameworks with rigorous controls and protocols in respect of protecting the dignity, privacy, and security of patients and citizens, with a strong focus on preventing issues from arising, and with response plans for data breaches. Such frameworks would include guidelines on legitimate use of data, especially on the commercialization of health data.

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