

## Biotechnology & Health:

### I. Breakthroughs in Biotechnology

#### Gene Editing Continues to Revolutionize Medicine:

**CRISPR-Cas9 Therapies Advancing:** Following the groundbreaking approval of Casgevy for sickle cell disease and beta-thalassemia, CRISPR-based therapies are rapidly expanding. Significant milestones in 2025 include the first successful patient-specific in vivo gene editing treatment for severe carbamoyl-phosphate synthetase 1 (CPS1) deficiency.

**Broader Applications:** The pipeline for CRISPR therapies is growing beyond monogenic diseases into oncology, viral infections, and autoimmune disorders. Research is also exploring base editing, prime editing, and RNA editing for even greater precision and safety.

**Ethical and Legal Landscape:** The ethical debates around germline editing and "designer babies" persist, alongside ongoing patent litigation, as seen in the ToolGen lawsuit against Vertex regarding Casgevy.

#### Cell and Gene Therapies (CGTs) – Towards Scalability and Accessibility:

**Allogeneic Therapies Gaining Ground:** A crucial trend is the shift towards allogeneic cell therapies (derived from healthy donor cells) to overcome the scalability and cost challenges of autologous therapies (using a patient's own cells). Innovations in manufacturing processes, including gene editing to reduce side effects like graft-versus-host disease, are making these "off-the-shelf" treatments more viable.

**Expanding Applications:** Allogeneic cell therapies are expanding beyond oncology to autoimmune diseases, cardiovascular disorders, and neurodegenerative conditions.

**CAR-T Therapies Evolving:** Chimeric Antigen Receptor T-cell (CAR-T) therapies continue to improve in efficacy and safety, with developments in controllable safety switches and combination approaches.

#### mRNA Therapeutics Beyond Vaccines:

Building on the success of mRNA-based COVID-19 vaccines, this technology is rapidly expanding. Researchers are exploring its applications in treating metabolic genetic diseases, cardiovascular conditions, and various forms of cancer, highlighting its versatility and relatively straightforward production process.

### II. Innovations in Health and Healthcare Delivery

#### AI's Transformative Role in Drug Discovery and Healthcare:

**Accelerated Drug Discovery:** AI is projected to generate billions in value for the pharmaceutical sector by 2025, with an estimated 30% of new drugs being discovered using AI. It speeds up the process by predicting compound effectiveness and safety, refining chemical structures, and optimizing target identification.

**Optimizing Clinical Trials:** AI is streamlining patient recruitment (e.g., using machine learning to analyze EHRs), making trial design more dynamic using real-world data (RWD), and enabling real-time data analysis to predict outcomes and adjust protocols. Generative AI is even being used to predict trial success rates.

### Enhanced Diagnostics and Precision Medicine:

AI algorithms are analyzing complex medical data for earlier disease identification, predicting protein structures (e.g., AlphaFold), and tailoring treatment plans based on genetic, molecular, environmental, and lifestyle factors. AI-powered diagnostic tools are advancing rapidly in areas like radiology and pathology.

### Precision Medicine - Tailoring Treatments to Individuals:

**Integration of Multi-Omics and AI:** The field of precision medicine is being driven by advances in AI, genomics, and data analytics. The combination of genomics, proteomics, metabolomics, and spatial omics provides deeper insights into individual biological profiles, enabling more personalized therapies.

**Personalized Diagnostics:** Genetic, proteomic, and metabolic data are being used for more accurate and earlier disease detection, reducing trial-and-error in treatment. Next-generation sequencing (NGS) is becoming more routine, even at the bedside, for rapid diagnosis of genetic conditions.

**Focus on Oncology:** Precision oncology continues to be a leading application, with comprehensive tumor profiling becoming standard practice to guide targeted therapies.

### Advancements in Vaccine Technology:

**Continued Focus on mRNA and Recombinant Vaccines:** These technologies remain central to vaccine development, with ongoing research into new products for various respiratory viruses (e.g., influenza, RSV), HSV, HIV, and even personalized or therapeutic vaccines (e.g., for cancer).

**AI in Vaccine Development:** AI is being increasingly used to speed up and improve vaccine development, from identifying vaccine targets and predicting immune responses to optimizing mRNA vaccine stability and selecting clinical trial participants.

**Data and Technology for Safety Monitoring:** There's a strong focus on using technology, such as data tokenization, to facilitate resource-efficient and long-term post-market surveillance of vaccine safety, addressing regulatory demands and public trust.

### Addressing Global Health Challenges:

**RSV Antiviral Drug Inclusion by WHO:** Ziresovir, developed by Shanghai Ark-Biopharmaceutical Co., Ltd. (ArkBio), has been included in the WHO's Paediatric RSV Drug Optimization (PADO-RSV) priority list. This is the first anti-RSV drug from China to receive this recognition, addressing an urgent clinical need for infants and young children globally.

**Alzheimer's Biomarker Research:** New studies are exploring the dual role of phosphorylated tau (p-tau217) in Alzheimer's disease and newborns, with surprisingly high levels found in newborns potentially linked to brain development. This opens new avenues for diagnostics and understanding.

**Addressing Noncommunicable Diseases (NCDs) and Mental Health:** A recent PAHO/WHO report estimates NCDs and mental health conditions will cost South America over US\$7.3 trillion in lost productivity and healthcare spending by 2050, highlighting the urgent need for prevention and integrated care.



**Wound Care Innovations:** Companies like Kane Biotech are presenting new developments in novel wound care treatments that disrupt biofilms.

**WHO's Global Health Agenda:** The Seventy-eighth World Health Assembly in May 2025 focused on key areas including robust regulation of digital marketing to protect children's health (especially formula milk), accelerating investment in health professionals, a new global traditional medicine strategy for 2025–2034, and a landmark resolution for a lead-free future. The WHO also noted that chronic diseases are the leading causes of death among people under 70 and emphasized the need to address global health inequities.

### MCQS

1. Which gene-editing therapy, already approved for sickle cell disease and beta-thalassemia, is mentioned as rapidly expanding in its applications?

- A) TALEN
- B) Zinc Finger Nucleases
- C) CRISPR-Cas9
- D) RNA interference

**Answer:** C) CRISPR-Cas9

The text states, "Following the groundbreaking approval of Casgevy for sickle cell disease and beta-thalassemia, CRISPR-based therapies are rapidly expanding."

2. What is a key trend in Cell and Gene Therapies (CGTs) aimed at overcoming the scalability and cost challenges of autologous therapies?

- A) Increased reliance on traditional chemotherapy
- B) Shift towards allogeneic cell therapies

C) Focus on developing more patient-specific autologous therapies

D) Reduction in the use of gene editing techniques

**Answer:** B) Shift towards allogeneic cell therapies

The text highlights, "A crucial trend is the shift towards allogeneic cell therapies (derived from healthy donor cells) to overcome the scalability and cost challenges of autologous therapies."

3. What percentage of new drugs are estimated to be discovered using AI by 2025, according to the text?

- A) 10%
- B) 20%
- C) 30%
- D) 50%

**Answer:** C) 30%

The text states, "AI is projected to generate billions in value for the pharmaceutical sector by 2025, with an estimated 30% of new drugs being discovered using AI."

4. What are "Multi-Omics" in the context of precision medicine, as described in the text?

- A) A combination of different types of medical imaging.
- B) The study of multiple patient symptoms for diagnosis.
- C) The combination of genomics, proteomics, metabolomics, and spatial omics.
- D) A method for analyzing environmental factors only.

**Answer:** C) The combination of genomics, proteomics, metabolomics, and spatial omics.

The text explains, "The combination of genomics, proteomics, metabolomics, and spatial omics provides deeper insights into



individual biological profiles, enabling more personalized therapies."

**5. Which company developed Ziresovir, the anti-RSV drug included in WHO's Paediatric RSV Drug Optimization (PADO-RSV) priority list?**

- A) Vertex Pharmaceuticals
- B) Kane Biotech
- C) Shanghai Ark Biopharmaceutical Co., Ltd. (ArkBio)
- D) Moderna

**Answer:** C) Shanghai Ark Biopharmaceutical Co., Ltd. (ArkBio)

The text states, "Ziresovir, developed by Shanghai Ark Biopharmaceutical Co., Ltd. (ArkBio), has been included in the WHO's Paediatric RSV Drug Optimization (PADO-RSV) priority list."

**6. A recent PAHO/WHO report estimates Noncommunicable Diseases (NCDs) and mental health conditions will cost South America how much in lost productivity and healthcare spending by 2050?**

- A) Under US\$1 trillion
- B) Over US\$7.3 trillion
- C) US\$100 billion
- D) US\$5.5 trillion

**Answer:** B) Over US\$7.3 trillion

The text mentions, "A recent PAHO/WHO report estimates NCDs and mental health conditions will cost South America over US\$7.3 trillion in lost productivity and healthcare spending by 2050..."

**7. What is one of the key areas of focus for the Seventy-eighth World Health Assembly in May 2025, concerning children's health?**

- A) Promoting unlimited digital marketing for all products.

B) Robust regulation of digital marketing to protect children's health (especially formula milk).

C) Decreasing investment in health professionals.

D) Discontinuing research into traditional medicine.

**Answer:** B) Robust regulation of digital marketing to protect children's health (especially formula milk).

The text states, "The Seventy-eighth World Health Assembly in May 2025 focused on key areas including robust regulation of digital marketing to protect children's health (especially formula milk)..."

**8. Beyond vaccines, what are some of the new applications being explored for mRNA therapeutics?**

- A) Only treatments for infectious diseases.
- B) Treating metabolic genetic diseases, cardiovascular conditions, and various forms of cancer.
- C) Primarily aesthetic medical procedures.
- D) Agricultural biotechnology.

**Answer:** B) Treating metabolic genetic diseases, cardiovascular conditions, and various forms of cancer.

The text states, "Researchers are exploring its applications in treating metabolic genetic diseases, cardiovascular conditions, and various forms of cancer..."

