Public Policy Challenges in Facilitating Access to Gene Therapy Medicines

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The transformative journey of gene therapy from a promising concept to a medical reality mark an extraordinary advancement in treatment possibilities, particularly for diseases lacking therapeutic options. The field's genesis was marked by the approval of the first clinical trial in 1990. Progress in genomic editing technologies and the discovery of RNA interference (RNAi) have since refined genetic engineering's precision and scope, leading to an expansion in the spectrum of treatable diseases. By 2023, this progress was evidenced by over 3,000 clinical trials, resulting in the registration of 30 gene therapy and 29 RNA therapy medicines, signaling the field’s rapid growth and potential.

Gene therapy has reached milestones such as curing certain monogenic diseases and inducing remission in advanced-stage cancer patients. However, the accessibility of these medicines is limited to a very small segment of the population, primarily due to the prohibitive costs arising from significant development investments and the complexity of production and validation processes. These factors create obstacles to widespread availability, making public health systems the only option in many countries.

The monopolization of gene therapy technologies by certain countries exacerbates access issues, as importation becomes the only option for many nations. The lack of generic versions of these medicines and the constraints imposed by intellectual property rights further hinder efforts toward local production and affordability.

Brazil has been working to address these public policy challenges. With considerable scientific and technological expertise in gene therapy, Brazil established foundational research and development with the Millennium Institute of Gene Therapy from 2005 to 2008, with the support of the National Council for the Development of Science and Technology (CNPq), conducting the first autochthonous gene therapy clinical trial and forming highly skilled professionals. The regulatory guidelines established by the Brazilian Health Regulatory Agency (ANVISA) for advanced therapy products since 2020, support this base. These advancements reflect Brazil’s commitment to adhering to best practices and international standards.

At the forefront of these initiatives is the National Institute of Gene Therapy, INTERGEN, founded in 2023 with the support of the CNPq, Capes and FAPESP. INTERGEN is committed to pioneering gene therapy research and promoting domestic product development and expertise. Its strategic objective is to collaborate with the government sectors to reduce the dependence on expensive imported gene therapy medicines, thereby easing the economic pressure on the public health system and aligning with the goal of making access to these innovative treatments more equitable.