Assessing Gene Therapy Innovation: Discovery, Clinical Development, Regulatory Authorization, and Market Dissemination

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Abstract

Background: Gene therapies represent a paradigm shift in the prevention and treatment of disease. The development of these therapies depends on complex interactions among publicly funded universities, hospitals, and research institutions, biopharmaceutical industry, regulatory agencies, and health care payers. The objective of this study is to assess the roles of the public-sector, universities, hospitals, and other research organizations, regulatory authorities, and the biopharmaceutical industry in gene therapy discovery, clinical development, regulatory authorization, and market dissemination.

Methods: We compiled data on all gene therapies authorized worldwide through December 15, 2025. Using regulatory records, bibliographic analysis, patent data, and clinical trial registries, we examined the scientific origins of gene therapies, characteristics of clinical development programs, regulatory designation and procedures expediting the development and authorization of these therapies by the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA) patent ownership patterns, and the worldwide dissemination of regulatory authorizations.

Results: There were 46 gene therapy products authorized by at least one regulatory agency as of December 15, 2025, with approximately two-thirds of authorization occurring between 2020 and 2025. Across the different gene therapy classes, foundational and basic science discoveries originated in publicly funded academic, research institutions and hospital decades prior to approval. The clinical development and regulatory review periods in the U.S. were a median (interquartile range-IQR) of 5.1 (4.6) years and 0.7 (0.3) years respectively. Half of the patents of gene therapies had universities, hospitals, and public institution assignees. The development and authorization of gene therapies facilitated by multiple designations and expedited pathway, including orphan designation granted by the FDA and EMA to.65.2.0% and 88.9%, respectively. Gene therapy authorizations occurred only in advanced economies and countries with large populations. The larger number of authorizations were granted by the FDA (n=27) and the EMA (n=23) The U.S. and the European Union, followed by Switzerland (12), China (11), and Canada (11),

Conclusions: The authorizations of gene therapies increased after 2020. Foundational scientific discoveries originated in publicly funded universities, hospitals, and research institutions. The combined clinical development and FDA review was under six years. Most therapies received multiple expedited regulatory designations, particularly orphan drug status. The diffusion of authorization was limited to high-income and large-population countries.