

FoCUS Project

Financing of Cures in the US

RESEARCH BRIEF

2017F211.v011

Existing Gene Therapy Pipeline Likely to Yield Dozens of Approved Products Within Five Years. A significant but not overwhelming number of gene therapy products will likely gain regulatory approval over the next five years. MIT NEWDIGS researchers in the FoCUS Project estimate that 39 (+/- 4) gene therapies will be approved by the end 2022 from the 2017 pipeline of 932 development candidates, including those already approved. 45% of the total are expected in oncology. High upfront reimbursement for these precision medicine products with durable, perhaps lifetime, effects may require precision financing approaches.

By **MIT NEWDIGS FoCUS Project**
13 November 2017

Gene therapies promise potentially durable clinical benefit from a single treatment course. They also promise high up-front costs for the extended, perhaps lifetime, benefits they confer. Estimating the number of gene therapies that will gain approval and launch in the five years can inform the planning by all stakeholders to ensure that patients gain appropriate access as rapidly as possible.

The MIT NEWDIGS consortium FoCUS Project (Financing and Reimbursement of Cures in the US) seeks to understand the financial challenges gene therapies may create and generate solutions to mitigate them. This multi-stakeholder effort gathers patient advocacy groups, payers from all segments of the US healthcare system, providers, regulators, and academics working in healthcare policy, financing, and reimbursement.

Understanding the productivity of the gene therapy pipeline is an important first step in projecting the

KEY TAKEAWAYS

1. About 40 gene therapies by 2022 will likely gain regulatory approval
2. Four gene therapy archetypes were identified
 - a. Oncology will see the most gene therapies (45%)
 - b. Novel Breakthroughs non-oncology orphan diseases for ultra small populations (4%).
 - c. Orphan Disrupters for remaining non-oncology orphan conditions (34%)
 - d. Budget Buster gene therapies in therapeutic areas such as cardiology, metabolic disorders and neurology may reach 1/5 (17%) of products.
3. Precision financing that targets the specific challenges of each payer and stakeholder type in each gene therapy archetype will be needed.

financial impact, challenges and solutions for each stakeholder.

Our analysis began with Informa/Citeline Pharmaprojects gene therapy clinical pipeline data from 2017.¹ We identified 925 products in development, across 209 indications. Figure 1 describes the distribution from pre-clinical through development phases to products under regulatory review.

Using published estimates of development phase transition rates for other therapeutics, since gene therapies do not yet have significant history, and FoCUS community estimates of phase durations and fractions of regulatory submission from each phase, we projected the approval probability and timing of each candidate product.

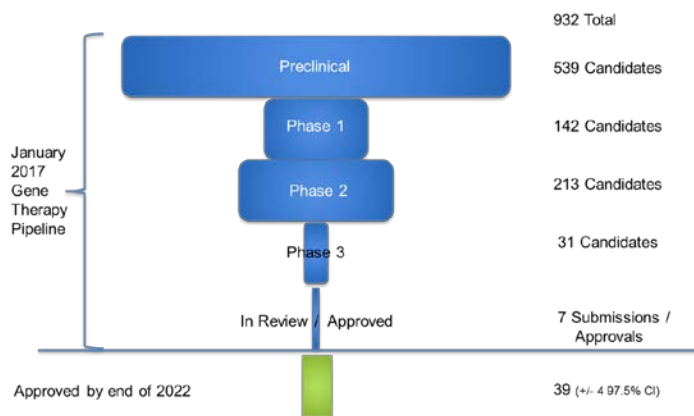


Figure 1 The pathway from preclinical to successful launch

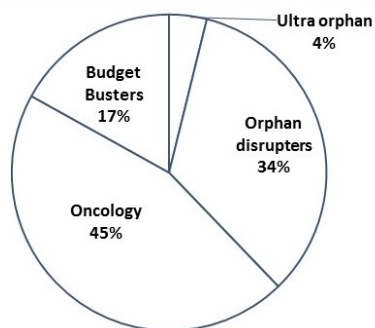


Figure 2 Product approvals by archetype through end of 2022

Five years from now, by the end of 2022, we estimate the existing, non-replenished gene therapy product pipeline will produce 39 approvals (+/- 4, 97.5% confidence interval). The concentration of products in preclinical lessens the likelihood of a large number near term approvals.

We further divided the product approvals into four gene therapy archetype (Figure 2). Members of each archetype share therapeutic area and patient population characteristics. Each archetype differs significantly from the others regarding the likely reimbursement approach that will apply and the financial challenges from volume and cost that they will create.

- **Oncology products (45%):** gene therapies to generate durable (>12 month) cancer patient response we estimate to be the largest number of products. In the past quarter alone, two products have been approved by the FDA, Kymriah™ by Novartis Pharmaceuticals and Yescarta™ by Gilead Sciences.
- **Novel breakthroughs (4% of 2022 approved products):** ultra-orphan with less than 100 new cases per year, non-oncology therapies, either chronic or acutely fatal
- **Orphan disrupters (34%):** larger in scale than novel breakthroughs, with less than 200,000 prevalent patients
- **Budget busters (17%):** indications with large prevalent populations, representing a high ‘surge’ effect for a new therapy in therapeutic areas such as cardiology, metabolic disorders, neurology and rheumatology.

Each archetype presents unique financial challenges for payers, patients, providers, developers and other stakeholders. The orphan archetypes present actuarial as well as performance (efficacy and durability) financial challenges. Oncology products enter an already high cost treatment context with multiple therapeutic alternatives. Budget busters, while a fewer number of products, enter indications with large populations that raise affordability concerns, especially if all prevalent patients medically qualify for immediate treatment.

Precision financing that targets the specific challenges of each payer and stakeholder type in each gene therapy archetype will be needed.

REFERENCE

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Please cite using MIT NEWDIGS Research Brief 2017F211.v011