

NEW DIGS

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Financing and Reimbursement
of Cures in the US

RESEARCH BRIEF

By MIT NEWDIGS
FoCUS Project
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Projections from the existing pipeline of cell and gene therapies: Launches and patient numbers

Our objective in this analysis was to estimate, at the indication level, durable cell and gene therapy new product launches in the US through 2030, and the number of potentially treatable patients.

Methods

Pipeline data for a range of cell and gene therapies were extracted from Informa Citeline/Pharmaprojects and further refined using clinicaltrials.gov and company web sites. Probabilities of success and estimates of clinical phase trial lengths were derived from the consolidated pipeline history and used to estimate future launches by indication and year. Estimates for treatment eligibility, including rates and timing of uptake for new patients and of clearance for patients with the disease were derived by our research team from publicly available data. Treatment eligibility was combined with existing epidemiological data on disease incidence and prevalence to generate estimated numbers of treatable patients.

KEY TAKEAWAYS

- Pipeline would be expected to lead to 40-60 launches, with 15-30 launching within the next five years.
- Projected that around 500,000 patients will have been treated with 40-60 products by 2030.
- Our analysis indicates that success rates and timelines for curative products differ substantially from previous industry experience.

Analysis

- Figure 1 provides a structural view of the modeling process.
- Each therapy for each indication is modeled individually with the results aggregated into therapeutic classes.
- Multiple indication products are modeled separately for each indication.
- Empirical data is drawn from completed clinical trials in cell and gene therapy.

Results

As an example of the trial completion data that were compiled, Figure 2 shows trial lengths for a sample of 71 completed (or terminated) phase 3 trials of cell and gene therapies. These data form the basis for the model's predictions of completion for individual trials.

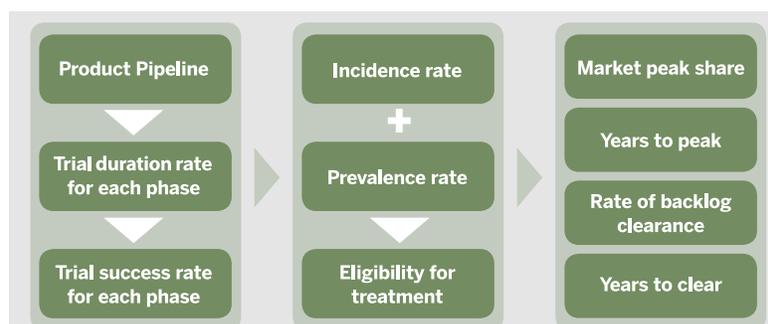


Figure 1. The pipeline analysis model

The Informa Citeline/Pharmaprojects database provided a pipeline of 661 active, individual US based clinical trials programs for durable cell and gene therapies comprising:

- 211 indications
- 232 preclinical

Based on our estimated success rates and trial lengths, this pipeline would be expected to lead to 40-60 launches (see Figure 3), with 15-30 launching within the next five years. Over the duration of our forecast around half the launches are expected to be in B-cell (CD-19) lymphomas and leukemias.

In total, we project that around 500,000 patients will have been treated with 40-60 approved therapies by 2030. In 2030, up to 100,000 patients per year may be treatable with cell and gene therapies (Figure 4). Estimates though are dependent on incidence in exactly which diseases have new products and, in oncology, assumes treatment of relapsed/refractory disease only.

CONCLUSION

The next 12 years will likely see significant changes in the US healthcare system with major improvements in the treatment paradigms for numerous diseases that previously had high morbidity and mortality. These will require the overall system to adapt, particularly in terms of how treatments are reimbursed and financed, as we move from chronic palliative therapies to acute curative ones. Our analysis (not shown here) suggests that success rates and timelines for curative products differ substantially from previous industry experience, although the historical information on these therapies is, of necessity, still somewhat limited. As a benchmark, the likelihood of approval from Phase I trials for all indications, according to Bio Reports data from 2006-2015, is 9.6%, whereas, we found this probability was found to be, on average, 5.0% for gene therapies and CAR-T and TCR therapies. Tools for precision financing need to be developed and in place so as to address these challenges when they arise.

ABOUT FOCUS

The MIT NEWDIGS consortium FoCUS Project (Financing and Reimbursement of Cures in the US) seeks to collaboratively address the need for new, innovative potentially curable therapies that ensure patient access and financing and reimbursement models for durable and sustainability for all stakeholders. Our mission is to deliver an understanding of financial challenges created by these therapies leading to system-wide, implementable precision financing models. This multi-stakeholder effort gathers developers, providers, regulators, patient advocacy groups, payers from all segments of the US healthcare system, and academics working in healthcare policy, financing, and reimbursement.

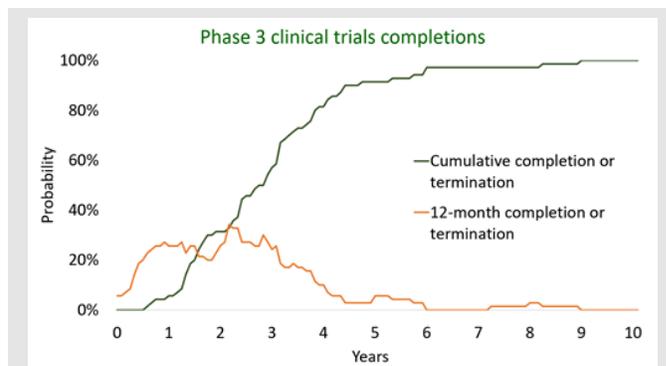


Figure 2. Predictions of Phase 3 clinical trial completion

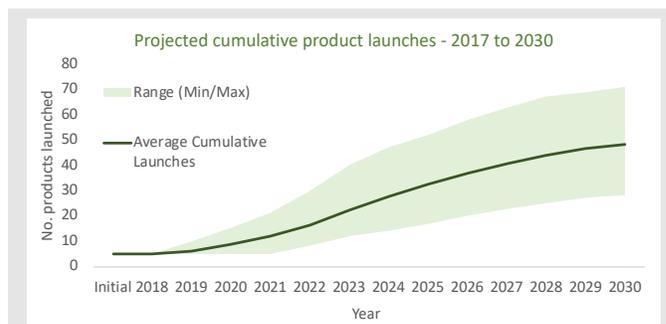


Figure 3. Predicted product launches, 2018-2030

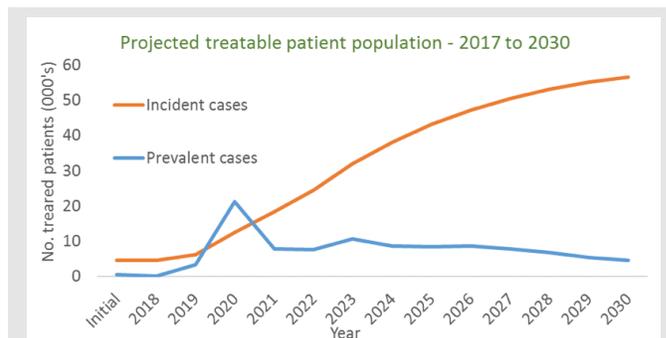


Figure 4. Predicted treated patient numbers, 2018-2030

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