KPMG Life Sciences

Experience In Cell & Gene Therapies

At KPMG, we are assisting clients working at the forefront of the next generation of therapies - cell and gene technologies. While these technologies are transforming patient care for certain diseases, they also bring their own unique complexities. KPMG brings a holistic view to the challenges and opportunities associated with the development, manufacturing, supply, pricing, and commercialization of these therapies. We continue to support clients, from small biotechs to large biopharma, contract research organizations (CROs), contract development and manufacturing organizations (CDMOs), and also private investors, to help them successfully negotiate these challenges and seize the opportunities that cell and gene therapies present.

Cell and gene therapies represent the next great frontier in medicine…

900

Number of gene therapy Investigational New Drug (IND) applications FDA stated had been submitted to the agency as of January 2020[3]

~1000

Companies worldwide working in regenerative medicine, including cell and gene, and tissue engineering[1]

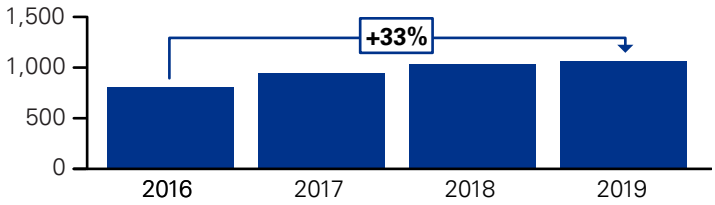
$600M

Size of the global cell and gene therapies market in 2016[2]

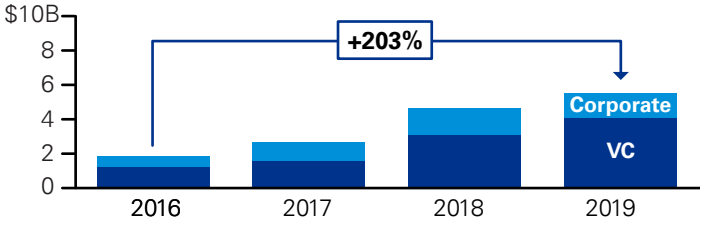
$62B

Forecasted size of the global cell and gene therapies market in 2026[2]

WW Ongoing Clinical Trials, 2016-2019[1]*



WW VC & Corporate Partnership Funding, 2016-2019[1]^



*Gene therapies, gene modified cell therapies, cell therapies, and tissue engineering; ^Corporate partnerships represent the value of upfront payments

…but hurdles still exist across the value chain

Development, Manufacturing & Supply

- Rapidly evolving market from a modality and delivery perspective leaving companies trying to understand **where to "place their bets".**
- Question marks over safety and efficacy of certain types of nucleic acid therapies such as "one-and-done" mean **the bar is high to prove safety and durability.**
- Many non-oncology nucleic acid markets are focused on rare monogenic diseases, which adds to **complexities in patient identification & recruitment for clinical trials.**
- A highly fragmented and immature raw material supplier ecosystem is leading to **manufacturing bottlenecks and impacting ability to scale supply.**
- Labor intensive manufacturing & high start-up costs, particularly with *ex vivo* therapies, **leading to high COGS.**
- Extremely rich valuations on acquisition targets are **pricing many companies out of the bidding.**

Regulatory & Commercialization

- Regulatory guidelines continue to evolve quickly, meaning companies must **keep pace to ensure they are compliant.**
- Payer systems are not mature enough yet to address ultra-high priced therapies, leaving companies to develop **new, innovative pricing models.**
- **Small patient populations** for rare monogenic diseases create unique challenges for commercial teams to navigate in order to drive sales.
- Critical to be **early to market** when competing in a "one-and-done" environment as these therapies will address the prevalent population, leaving just the incident population.
- Given small clinical trials and continued concerns over long-term efficacy and safety, companies need to **commit to extremely long Phase IV trials.**

Examples only, not exhaustive
Sources: (1) Alliance for Regenerative Medicine, (2) EvaluatePharma, (3) FDA, (4) Pharmaprojects

KPMG's experience assisting clients in developing cell & gene therapies

Strategy

- Assessing modalities and delivery technologies to support R&D and business development strategy.
- Helping clients gain a competitive advantage by developing innovative market entry and post-launch strategies.
- Developing actionable strategic insights to support launch of new therapies into rare and ultra-rare genetic diseases.
- Assessing the size of market opportunities in cell and gene markets.
- Designing innovative pricing models to drive market access in key countries.
- Designing and developing financial and tax operating models.

Technical Operations

- Supporting clients in optimal organizational design and functional integration following acquisitions.
- Designing cross functional operating models and detailed roadmaps for "make-to-order" cell therapies.
- Analyzing end-to-end supply chain processes, systems, organization, and metrics to reduce lead times.
- Developing manufacturing strategies that are aligned with product characteristics and patient needs.

Deals

- Identifying inorganic growth opportunities for organizations and executing full diligence on targets.
- Conducting market landscape assessments for private investors looking for investment opportunities and conducting full diligence on identified targets.
- Supporting organizations as they look to develop strategic partnerships with raw material suppliers.

We engage clients across functions to support their strategic, operational & deal needs in cell and gene therapy

R&D | Manufacturing & Supply Chain | Medical Affairs | Business Development | Pricing & Market Access | Sales & Marketing

Example Case Studies:

We have successfully delivered dozens of projects in cell and gene therapy for both private investors and corporate clients

Strategy

- Mapped out the pre-launch and post-launch team structures and required skill sets for an upcoming gene therapy launch for a biopharma client.
- Supported a large biopharma client in development of an innovative pricing model for its "one-and-done" gene therapy.
- Strategic roadmap ("where to play" and "how to win") for a biopharma client looking to enter the cell and gene therapy market in both neurology and in an ultra-rare immunology disease.

Technical Operations

- Analysis of supply chain issues for a biopharma client's autologous program, and identification of market feasible approaches to reducing supply-chain related total turnaround time.
- Designed and implemented cross-functional operating model, business processes and technologic capabilities to support end-to-end autologous CAR-T therapy.

Deals

- Diligence for a global biopharma on a target developing an *ex vivo* HSCT gene therapy, focusing on the manufacturing, clinical and commercial considerations for successful integration of the target.
- Commercial due diligence for a biopharma client on a target developing "one-and-done" gene therapies in neurological and blood disorders.
- Market landscape assessment of the cell and gene raw material suppliers for a private equity client

Why KPMG?

We Have Unparalleled Experience

Our teams have worked with clients across the cell and gene therapy value chain, which means we bring deep experience and strategic insights to each project.

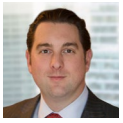
We Work Across Client Types

We work across client types, from the smallest biotech to the largest biopharma and across the private equity continuum, meaning that we can provide our clients with unique perspectives.

We Bring Global Strength

The KPMG network has local teams around the world that understand the nuances of their markets. We are able to bring these local insights to bear, while working as a global team, to ensure project success.

Kristin Pothier
Global and National Healthcare and Life Sciences Leader, KPMG Strategy



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