Commentary: Paige Bischoff

A record year for the financing of ATMPs

In the midst of a global pandemic, the world cheered the unprecedented development and roll-out of several effective Covid-19 vaccines. Although it did not receive the same fanfare, the regenerative medicine sector had an equally impressive 2020, with scientific advancements that have the potential to advance human health for years to come.

Advanced therapy medicinal products (ATMPs) – which include cell, gene and tissue-based therapies – achieved the greatest financing year on record, despite all the hurdles that 2020 presented. Our team at the Alliance for Regenerative Medicine (ARM) is pleased to provide readers of *MedNous* with a first look at our full year 2020 data for the sector.

The ATMP story is one of scientific progress along the continuum of clinical trials, across both rare and more prevalent diseases, and through a variety of technological approaches that have turned theory into practice. These factors have coalesced to attract strong interest from investors in venture capital, public capital markets, and beyond. At ARM we are dedicated to promoting sound regulatory, reimbursement and policy priorities which will ensure that this immense promise translates into transformative treatments for patients around the world.

Global financing for the regenerative medicine sector reached $19.9 billion in 2020, eclipsing the previous record of $13.5 billion set in 2018. Taken together, publicly traded regenerative medicine companies saw a roughly 50% increase in performance in 2020, outperforming the NASDAQ Biotechnology Index. What is particularly impressive is that the ATMP sector exceeded its previous financing record by the end of the third quarter, when we reported total financings for the first nine months of 2020 of $15.9 billion.

Financing was robust across advanced therapy technologies. As in previous years, gene therapy continues to form the largest segment, with $14.7 billion raised to date, an increase of 73% from 2019. Cell therapy is close behind at $13.2 billion total financings, a rise of 160% year-on-year. Tissue-based therapy, a smaller sub-sector of ATMPs, raised $312 million in 2020.

The market for initial public offerings (IPOs) of healthcare stocks was buoyant in 2020, and ATMPs were no exception. IPOs and follow-on financings were responsible for $9.7 billion, and private financing for $10.2 billion.

Even with the backdrop of a global pandemic, investors remained confident about the promise of profound, durable and potentially curative regenerative medicines to treat a range of diseases and disorders.

The breadth of innovation now available, from CRISPR to chimeric antigen receptor T cells (CAR T) to lentiviruses and non-viral gene therapies, is enabling developers to target a broader range of tumours, genetic mutations and damaged tissues – with increasing support from financial backers.

The record financing of 2020 also comes at a time when regenerative medicine is showing progress across all stages of the clinical pipeline, and when more developers – with greater geographic diversity – are becoming active in the space.

At the end of 2020, there were 1,220 regenerative medicine clinical trials ongoing worldwide - including 158 in Phase 3. In total, there are 1,085 regenerative medicine and advanced therapy developers active across the globe, up from 987 in 2019. While the US and Europe remain the major hubs, East Asia is an increasingly significant region for ATMPs, and developers are also making progress in South America, South Asia, Australasia and Africa.

The range of indications being targeted in clinical trials are broad – another key draw for investors. A large proportion - 562 trials - are looking at oncology. However, there are also ongoing trials in areas including neurodegenerative diseases and immune deficiencies. Even with the arrival of Covid-19 vaccines, there are still 37 trials ongoing focused on the virus and its complications.

Regulatory decisions are expected on multiple product candidates in the coming months and years. These include anticipated near-term decisions from both the European Medicines Agency and the US Food and Drug Administration on Breyanzi (Bristol Myers Squibb) for relapsed or refractory large B-cell lymphoma, and Ide-cel (bluebird bio and BMS) for relapsed or refractory multiple myeloma. Both the FDA and EMA have said they each anticipate between 10 and 20 applications or approvals each year by 2025.

As we begin 2021 bolstered by this exceptional growth, healthcare systems need to fully prepare for the coming wave of ATMP approvals, in order to deliver the ultimate end goal - getting these treatments to patients. As clinical trials progress, data is demonstrating the transformative potential of ATMPs. Such treatments offer hope for patients with significant unmet needs across a huge range of disease areas, and as each year passes our understanding of the science improves and the durability of these therapies becomes more evident.

This commentary was written by Paige Bischoff, senior vice president of global public affairs at the Alliance for Regenerative Medicine.

