

**ADVANCED THERAPIES  
IN THE SPOTLIGHT**  
AT THE CELL & GENE MEETING  
ON THE MED

The transformative potential of regenerative medicine was in the spotlight at the inaugural Cell & Gene Meeting on the Mediterranean in Barcelona, Spain, held on 23-25 April. The conference, organised by the Alliance for Regenerative Medicine (ARM), was supported by Consilium Strategic Communications, event Media Partner and ARM's retained communications advisor for Europe.

The conference brought together the cell and gene therapy community from Europe and beyond to discuss ongoing progress and how best to turn the promise of regenerative medicines, or advanced therapeutic medicinal products (ATMP), into a reality that benefits patients and healthcare systems.

**Janet Lambert, Chief Executive Officer of ARM**, opened the event by summarising the scale of the regenerative medicine industry in Europe and its increasing maturity, with four advanced therapy approvals expected in the relatively near term: bluebird bio's Zynteglo, Kiadis Pharma's ATIR01, Axexis/Novartis's Zolgensma and Orchard Therapeutics' OTL-200.

This maturity brings great opportunities for patients but also challenges, many of which were discussed during the conference.

In his keynote address, **Guido Rasi, Director General of the European Medicines Agency**, stated that the "expectations for these medicines are enormous", and highlighted the tailored regulatory systems in place in Europe and the progress being made towards getting more therapies approved. As this continues, Rasi said, there will need to be new paradigms on evidence generation and reassuring regulators, owing to the small patient numbers in clinical trials. Rasi invoked a "never ending trial" with an initial approval that is later adjusted through the accumulation of real-world evidence.

However, despite confidence in the ability of regulators to overcome obstacles and adapt, Rasi raised what would be a key theme of the conference – the challenge of patient access and reimbursement. "It is essential that we don't become a body that approves a drug no one can access."

**Jeff Walsh, Chief Strategic Officer of bluebird bio**, shared his view that the ATMP sector has overcome multiple hurdles in science and manufacturing, but the biggest remaining challenges are those of commercial access. "How do you create an environment where patients can get access to these therapies in a robust way, and yet still allow for the value proposition to have legs?"

Accepting this was not an "insignificant challenge", Walsh, however, expressed confidence that due to the significant positive effects of cell and gene therapies on patient lives, "we'll figure this one out".

Other key themes to emerge were the

**...CONTINUED**

**"Of 904 regenerative medicine companies worldwide, 233 are in Europe and Israel and there are more than 324 active ATMP clinical trials, including 50 in Phase III.** The financing environment is also strong, according to ARM's figures, with **€488.3M raised in Q1 2019** (up 143% vs Q1 2018)."

**MONTHLY INTELLIGENCE****ANALYST MOVES**

- **Charles Weston** has started at RBC Capital Markets
- **Emma Ulker** has started at Proactive Investors as an Investment Analyst

**MEDIA MOVES**

- **Marie Powers** has retired from BioWorld



## CONTINUED...AMTPS IN THE SPOTLIGHT

complexity and critical importance of manufacturing for ATMPs and also how the industry and wider healthcare system adapts to the change from a focus on therapies that address small patient populations toward larger, more complex indications. **John Dawson, Chief Executive Officer of Oxford BioMedica**, which is the only FDA approved provider of commercial lentivectors, commented: "We are increasing our capacity levels, doubling in the short term and probably more in the future, to meet the expected demand and address the challenge of larger indications that need more vectors delivered."

On a panel entitled Recent Developments and Predictions for the Future of Advanced Therapies, **Amy DuRoss, Chief Executive Officer and Co-Founder of Vineti**, talked about the opportunity for collaboration to build the infrastructure for increasing numbers of therapies, stating that the US market is effectively operating as a "beta version" which could be developed and improved to move into other markets in the world.

This was an idea backed up by **Sven Kili, former Head of Cell & Gene Therapy Development at GSK**. He believes that in addition to collaborating to resolve reimbursement and infrastructure issues, the sector should work together to develop core standards. This way, as ATMPs go from treating tens of patients to thousands, tens of thousands or even millions, companies will be able to draw on set, accepted methods

and also avoid the risk of having standards imposed from outside.

The importance of collaboration in technical progression and integration was highlighted by **Debra Bowes, Chief Business Officer of MaxCyte**. Bowes explained how improvements were continually being made to therapeutic processes, product outcomes (the product being patients' cells) and, crucially, the reduction in time from identifying the patient to extracting their cells and returning them once modified – a key feature of MaxCyte's CARMA platform. She was optimistic, but concluded that there was much hard work left to do: "We're all working and collaborating to improve those processes, but we've got a way to go."

Whilst the challenges faced by developers were well recognised, the overall tone of the conference was one of optimism for the industry and for the impact on regenerative medicine, as summed up by **Shane De Bhruin-Smith, healthcare analyst at Cannaccord Genuity**. Talking about potentially curative treatments in oncology and other diseases, he said: "I think the impact of these therapies on patients and their families is very profound... from all stakeholders and all perspectives, that's tremendously exciting."

*Consilium, in collaboration with Brandcast Media, interviewed multiple key figures in the ATMP industry and these videos are available on the ARM Twitter account (@alliancernm).*

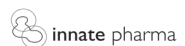


PROUD TO BE A SPONSOR  
**EUROPEAN  
MEDISCIENCE  
AWARDS 2019**



THURSDAY 13 JUNE  
THE BREWERY, LONDON EC1

CONSILIUM IS PROUD TO SEE A NUMBER OF OUR  
CLIENTS ON THE SHORTLIST FOR THE  
2019 MEDISCIENCE AWARDS



If you would like to receive our Monday Healthcare Bulletin, which summarises all healthcare business news from the weekend, please contact: [adminsc@consilium-comms.com](mailto:adminsc@consilium-comms.com)



## THIS MONTH'S MOVERS AND SHAKERS

- Immunocore appointed **Dr Mohammed Dar** as Head of Clinical Development and CMO
- Silence Therapeutics appointed **Iain Ross** as Chairman of the Board of Directors and **James Ede-Golightly** as a Non-Executive Director and **Dr Rob Quinn** as CFO
- Cambridge Innovation Capital announced that **Dr Sohaib Mir** was promoted to Principal within the Investment Team
- Sandoz appointed **Richard Saynor** as CEO, effective 1 August 2019
- Aduro Biotech appointed **Dimitry Nuyten** as CMO
- Paragon Biosciences appointed **James Robinson** as COO
- WindMIL Therapeutics appointed **Donald Hayden** as permanent CEO and Executive Chairman
- Translate Bio appointed **Richard Wooster** as CSO and **Robert Plenge** as Board Director
- X4 Pharmaceuticals appointed **E. Lynne Kelley** as CMO
- CANbridge Pharmaceuticals has appointed **Glenn Hassan** as CFO and CBO and **Gerald Cox** as chief development strategist and interim CMO
- Arrakis Therapeutics appointed **Michael Gilman** as full-time CEO
- Obsidian Therapeutics appointed **Paul Wotton** as CEO
- Kintai Therapeutics appointed **Paul-Peter Tak** as CEO
- Insys Therapeutics appointed current CFO **Andrew Long** as CEO
- Talaris Therapeutics, previously Regenerex appointed **Scott Requadt** as CEO
- Aurinia Pharmaceuticals appointed **Peter Greenleaf** as CEO
- Cyclerion Therapeutics appointed **Andy Busch** as Chief Innovation Officer
- NetScientific plc appointed **Ian Postlethwaite** as CEO in addition to his current position of CFO



## HIGHLIGHTED FUNDRAISING ACTIVITY FROM APRIL TO MAY 2019

COMPANY NAME	AMOUNT RAISED	TYPE OF FUNDRAISE	COUNTRY	DATE
<b>PUBLIC COMPANY FINANCINGS</b>				
<b>Evoform Biosciences</b>	\$80 million	Private placement	US	11-April-19
<b>Turning Point</b>	\$166.5 million	IPO	US	17-April-19
<b>Hookipa Pharma Inc.</b>	\$84 million	IPO	US	18-April-19
<b>Gritstone Oncology Inc.</b>	\$74.8 million	Follow-on	US	25-April-19
<b>Novus Therapeutics</b>	\$10.7 million	Registered direct	US	30-April-19
<b>Mustang Bio Inc.</b>	\$27.5 million	Follow-on	US	1-May-19
<b>Repligen Corp.</b>	\$175 million	Follow-on	US	1-May-19
<b>Biocartis Group NV</b>	€125 million	Convertible bond	Belgium	2-May-19
<b>Magenta Therapeutics Inc.</b>	\$56.3 million	Follow-on	US	2-May-19
<b>Zai Lab Ltd.</b>	\$200 million	Upsised offering	US	3-May-19
<b>Athenex Inc.</b>	\$100 million	Private placement	US	6-May-19
<b>PRIVATE COMPANY FINANCINGS</b>				
<b>Nocion Therapeutics Inc.</b>	\$27 million	Series A	US	17-April-19
<b>APathAI</b>	\$60 million	Series B	US	17-April-19
<b>Arrakis Therapeutics Inc.</b>	\$75 million	Series B	US	18-April-19
<b>Talaris Therapeutics</b>	\$100 million	Series A	US	18-April-19
<b>Poseida Therapeutics Inc.</b>	\$142 million	Series C	US	22-April-19
<b>BostonGene Corporation</b>	\$50 million	Series A	US	24-April-19
<b>Sirnaomics Inc.</b>	\$47 million	Series C	Hong Kong	29-April-19
<b>Alentis Therapeutics AG</b>	\$12.3 million	Series A	Switzerland	30-April-19
<b>Vividion Therapeutics Inc.</b>	\$82 million	Series B	US	30-April-19
<b>PolyProx Therapeutics</b>	£3.4 million	Seed financing	UK	30-April-19
<b>Congenica</b>	€23.3 million	Series B	US	30-April-19
<b>Spiral Therapeutics Inc.</b>	\$5.6 million	Series A	US	6-May-19
<b>Promethera Biosciences S.A.</b>	\$44.4 million	Series D	Belgium	7-May-19
<b>Confo Therapeutics</b>	€30 million	Series A	Belgium	7-May-19
<b>Verve Therapeutics</b>	\$58.5 million	Series A	US	7-May-19
<b>Repair Biotechnologies</b>	\$2.15 million	Seed financing	US	7-May-19
<b>Elligo Health Research</b>	\$20 million	Series C	US	7-May-19



## 2019

### MAY

- BioEquity Europe**, Barcelona (20-21 May)
- UBS Global Healthcare Conference**, New York (20-22 May)
- RBC Capital Markets 2019 Healthcare Conference**, New York (21-22 May)

### JUNE

- BIO International Convention**, Philadelphia (3-6 June)
- Jefferies 2019 Global Healthcare Conference**, New York (4-7 June)
- Goldman Sachs 40th Annual Global Healthcare Conference**, Palos Verdes (11-13 June)
- Bryan Garnier 4th Annual Oncology Day**, Paris (17 June)
- Citi 8th Annual European Healthcare Conference**, London (18-19 June)
- Next Generation CAR & T Cell Therapies**, San Francisco (18-20 June)
- JMP Securities Life Sciences Conference**, New York (19-20 June)
- JP Morgan European Healthcare Conference**, London (20 June)
- Piper Jaffray Heartland Summit**, Minneapolis (20 June)

### JULY

- LSE Future of Healthcare Investment Forum**, London (10 July)

### AUGUST

- Canaccord Genuity Annual Growth Conference**, Boston (7-8 August)

### SEPTEMBER

- Goldman Sachs Annual European MedTech & Healthcare Services Conference**, London (4-5 September)
- RW Baird Healthcare Conference**, New York (4-5 September)
- Wells Fargo Securities Healthcare Conference**, Boston (4-5 September)
- Goldman Sachs Annual Biotech Symposium**, London (7 September)
- Cell & Gene Therapy Manufacturing and Commercialization**, Boston (9-12 September)
- Boston BioTech BD Conference**, Boston (10 September)
- CAR-TCR Summit Boston 2019**, Boston (10-13 September)
- BAML Global Healthcare Conference**, London (10-13 September)
- Citibank Annual Biotech Conference**, Boston (11-12 September)
- Morgan Stanley Global Healthcare Unplugged Conference**, Boston (11-13 September)
- Sachs Annual Biotech in Europe Forum**, Basel (25-25 September)
- Rodman & Renshaw Annual Global Investment Conference**, New York (TBD)
- BioCentury Annual Newsmakers in the Biotech Industry**, New York (TBD)

### OCTOBER

- ARM Cell & Gene meeting on the Mesa**, Carlsbad (2-4 October)
- LSX World Congress USA**, Boston (8-9 October)
- BIA UK Bioscience Forum**, London (12 October)
- Sachs Annual Medtech & Digital Health Forum**, Basel (16 October)
- BIO Investor Forum 2019**, San Francisco (22-23 October)
- 10th Galien Forum**, New York (24 October)
- Cowen & Co. Annual Therapeutics Conference**, New York (25-26 October)
- Cantor Global Healthcare Conference**, New York (23-25 September)
- Ladenburg Thalman Healthcare Conference**, New York (TBD)

### NOVEMBER

- FT Global Pharmaceutical and Biotechnology Conference 2019**, London (11-12 November)
- Credit Suisse Annual Healthcare Conference**, Scottsdale (11-13 November)
- BIO-Europe 2019**, Hamburg (11-13 November)
- Bryan Garnier 2019 Healthcare Conference**, Paris (12-13 November)
- Stifel Nicolaus Weisel Healthcare Conference 2019**, New York (13-14 November)
- Consilium Strategic Communications Annual Healthcare Conference**, London (19 November)
- Inv€stival Showcase**, London (19 November)
- Jefferies Healthcare Conference**, London (20-21 November)
- Canaccord Genuity Medical Technology and Diagnostics Forum**, New York (21 November)
- Piper Jaffray Annual Healthcare Conference**, New York (27-29 November)

### DECEMBER

- Berenberg Bank Annual European Conference**, Surrey (2-5 December)
- Forbes Healthcare Summit**, New York (4-5 December)
- Citibank Global Healthcare Conference**, New York (5-6 December)

## 2020

### JANUARY

- 3rd Annual Neuroscience Innovation Forum**, San Francisco (12 January)
- BioTech Showcase 2020**, San Francisco (13-15 January)
- JP Morgan Annual Healthcare Conference**, San Francisco (13-16 January)
- Annual Needham Growth Conference**, New York (TBD)