



Q2

ALLIANCE_{for}
Regenerative Medicine

2017

Quarterly Data Report

on gene and cellular therapies and the regenerative medicine sector



ALLIANCE_{for} *Regenerative Medicine*

The Alliance for Regenerative Medicine (ARM) is the preeminent global advocate for regenerative and advanced therapies. ARM fosters research, development, investment and commercialization of transformational treatments and cures for patients worldwide.

By leveraging the expertise of its membership, ARM empowers multiple stakeholders to promote legislative, regulatory and public understanding of, and support for, this expanding field.

www.alliancerm.org

Table of Contents



| | |
|--|-------|
| <i>Global Landscape</i> | 2 |
| <i>Industry Overview</i> | 3 |
| <i>Financings</i> | 4-5 |
| <i>Commentary: Therapeutic iPSCs</i> | 6-7 |
| <i>Clinical Trials</i> | 8 |
| <i>Commentary: Reimbursement & Market Access</i> | 9 |
| <i>Major Milestones & Key Data Events</i> | 10-11 |
| <i>Current Regulatory & Legislative Priorities</i> | 12 |

Global Landscape

822+

Regenerative Medicine Companies Worldwide,
Including Gene and Cell Therapies

228

Europe
& Israel

122

Asia

435

North
America

15

South
America

1

Africa

21

Oceania
(Australia, New Zealand,
Marshall Islands)

Industry Overview

High expectations and persistent progress in advanced therapies continued across the sector in the second quarter of 2017.

Numerous companies pursued the FDA's new Regenerative Medicine Advanced Therapy (RMAT) designation, which facilitates expedited reviews and approvals for regenerative medicine products. Following Humacyte's lead, Enzyvant in April received not only the RMAT designation, but also Breakthrough Therapy designation for RVT-802, a biologic initially being tested for DiGeorge syndrome. This announcement was swiftly followed by iCyte and Vericel, each receiving their own RMAT designations: iCyte for its JCell product to treat retinitis pigmentosa, and Vericel for ixmyelocel-T for advanced heart failure due to ischemic dilated cardiomyopathy.

Organizations formed partnerships at a steady clip throughout the quarter. After the FDA granted orphan drug and Fast Track status to Sangamo Therapeutics' cDNA gene therapy for hemophilia A, the company entered into a deal worth up to \$545 million with Pfizer. In addition, Celyad signed a \$96 million deal with Novartis for non-exclusive licenses involving Celyad's allogeneic TCR-deficient CAR-T cells patents. Other notable deals in Q2 included Servier and Transgene's \$34 million agreement to produce allogeneic CAR-T cell therapies, Evotec's collaboration with Censo Biotechnologies patient-derived induced pluripotent stem cells, Sarepta and Genethon's gene therapy tie-up to study Duchenne's muscular dystrophy and Inovio's agreements with both Genentech and Regeneron to partner the company's T cell activating immunotherapies with checkpoint inhibitors.

In addition, a number of significant public offerings this quarter highlighted continuing investor confidence in regenerative medicine approaches to treating disease. bluebird bio announced the pricing of a secondary offering, hoping to raise an estimated \$400 million. AveXis raised almost \$270 million, which it plans to use for clinical trials of AVXS-101 in SMA and Sangamo Therapeutics pulled in \$83.4 million in a secondary offering, close to the amount REGENXBIO raised closing its public offering at \$87.2 million. The quarter's sole IPO belongs to Tocagen, who raised \$97.8 million to advance its cancer gene therapy platform.

Several progressive acquisitions took place in Q2, including deals that will lead to further manufacturing opportunities and therapy development. Cell Medica acquired Catapult Therapy TCR and established collaboration with the UK's Cell and Gene Therapy Catapult to manufacture cell therapies. Hitachi Chemical acquired the remaining 80.1% stake of PCT from Caladrius Biosciences, which will greatly expand Hitachi's expertise in cell therapies and manufacturing on a global level. Heat Biologics acquired Pelican Therapeutics, Lonza bought PharmaCell BV, a CMO for cell therapies and regenerative medicine and Intrexon acquired GenVec for their expertise in adenovirus gene delivery technology.

Looking ahead, the U.S. FDA Advisory Committee's recent unanimous vote in favor of approval for Novartis's anti-CD19 CAR-T product for the treatment of pediatric and young adult patients with relapse/refractory B-cell acute lymphoblastic leukemia sets the stage for an eventful and possibly historic third quarter, with perhaps several highly anticipated first-in-the-U.S. product approvals. In addition to Novartis' product, this likely includes Kite Pharma's CAR-T therapy for aggressive non-Hodgkin lymphoma and Spark Therapeutics' LUXTURNA gene therapy for inherited retinal disease, marking major milestones for the sector.

-Patricia Reilly
Head of Intelligence Alliances and Unification
Pharma Intelligence

-Nancy Dvorin
Managing Editor, IN VIVO, Start-Up and Medtech Insight

Informa Business Intelligence, Pharma and Healthcare



Total Q2/1H 2017 Global Financings



TOTAL GLOBAL FINANCINGS

\$2.45 Billion
raised in Q2 2017

88% increase from Q2 2016

\$4.25 Billion
raised in 1H 2017



GENE & GENE-MODIFIED CELL THERAPY

\$1.18 Billion
raised in Q2 2017

56% increase from Q2 2016

\$2.04 Billion
raised in 1H 2017

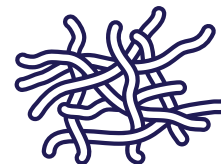


CELL THERAPY

\$1.33 Billion
raised in Q2 2017

70% increase from Q2 2016

\$2.71 Billion
raised in 1H 2017



TISSUE ENGINEERING

\$90.7 Million
raised in Q2 2017

64% increase from Q2 2016

\$319.8 Million
raised in 1H 2017

Examples of Key Financings: Q2 2017

Public offerings:

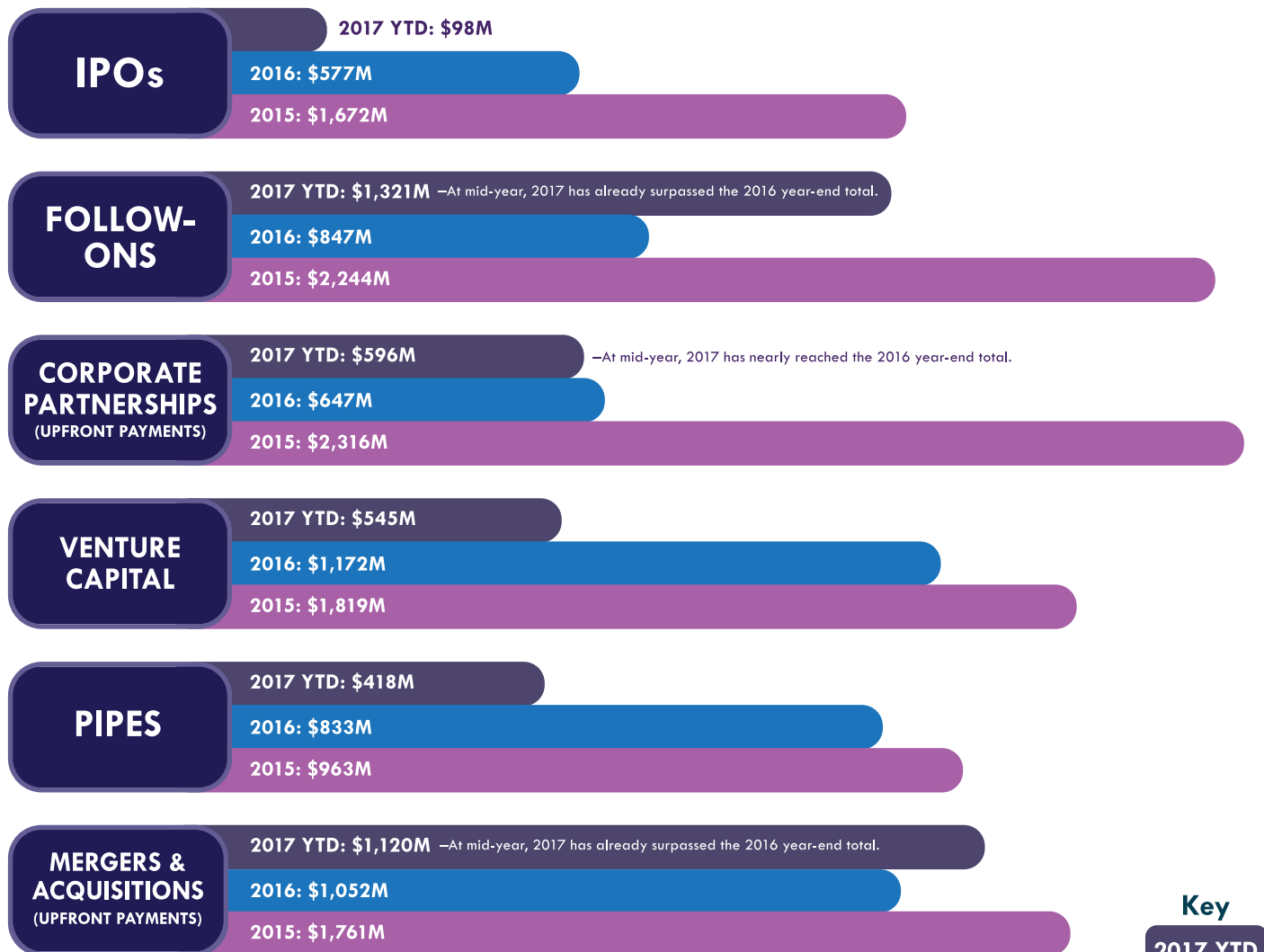
- AveXis raises \$269.8M in public offering of common stock – June 26, 2017
- Tocagen raises \$97.8M in initial public offering – April 19, 2017
- REGENXBIO raises \$87.2M in public offering of common stock – April 26, 2017
- Audentes Therapeutics raises \$86.3M in public offering of common stock – April 24, 2017
- Sangamo Therapeutics raises \$83.4M in public offering of common stock – June 26, 2017

Corporate partnerships, acquisitions and other financings:

- Sanpower Group completes Dendreon acquisition from Valeant for \$819.9M – June 29, 2017
- Sangamo Therapeutics signs \$545M hemophilia A gene therapy collaboration with Pfizer, including \$70M upfront – May 10, 2017
- Takeda signs \$100M agreement with GammaDelta Therapeutics to develop novel gamma delta T cell platform – May 9, 2017
- Rubius Therapeutics raises \$120M in private financing – June 21, 2017
- Celyad signs \$96M agreement with Novartis for allogeneic TCR-deficient CAR-T cells patents – May 2, 2017
- Hitachi Chemical acquires the remaining 80.1% stake in PCT from Caladrius Biosciences for \$80M – May 18, 2017

**Total amount raised represents sector-wide figures; please note that some companies utilize technology from more than one technology group. As a result, the total financings amount does not equal the sum of the raises of the individual technology groups.*

Total Global Financings by Type, by Year



Key

2017 YTD

2016

2015

Amounts in USD.

Commentary: Therapeutic iPSCs

| | | | | | | |
|----------------------|---|--|--|---|--|--|
| Commentators: | BlueRock Therapeutics Emile Nuwaysir President & Chief Executive Officer | Cellular Dynamics International, a FUJIFILM Company Lucas Chase Director, Ocular Cell Therapy | Peter Fuhrken Group Leader, Process Sciences | Derek Hai Vice President, Clinical Manufacture, Quality and Regulatory | Amanda Mack Director, iPSC Reprogramming | Christopher McMahon Senior Group Leader |
|----------------------|---|--|--|---|--|--|

1) What is your organization's view regarding last December's passage of the 21st Century Cures Act and the bill's new RMAT designation for cell therapy products? What significance does this have for the cell therapy sector overall?

The new RMAT designation creates a regulatory path that will allow greater interactions with the FDA during cell therapy development potentially resulting in an accelerated path to approval. This new designation could have a significant positive impact on moving promising new cell therapeutics through the FDA approval process. – DH

This is a very positive step for cell therapy in the U.S. It will get therapies to the market more quickly, and will foster rapid innovation in the field. Although of course there is a balance between safety and speed, these types of reforms will reduce unneeded delays, in particular by allowing trials to proceed based on intermediate or surrogate endpoints. – CM

2) There appears to be a growing interest in induced pluripotent stem cells (iPSCs) as therapeutic platforms to treat CV disease, neurodegenerative disease and other indications. What advantages do iPSCs offer?

Unlike many multipotent and pluripotent human stem cells, iPSC technology enables the generation of patient-specific (autologous) or patient-compatible (HLA matched) pluripotent stem cells. As such, it becomes possible to generate therapeutically relevant cell types in a manner that is compatible with a patient's immune system. Also, iPSCs can be generated from various adult cell sources, including peripheral blood, skin and many others. This ability to utilize an adult cell type as a source for iPSCs avoids the potential ethical issues associated with embryonic or fetal stem cells. – LC

The use of iPSCs minimizes the need to continually seek donors for tissue samples. iPSCs offer a renewable material source on which to build information through experimental replicates as well as examine performance and safety. When primary cells are used, for example, their numbers are often limited, minimizing the amount of information gathered before the need to obtain additional material arises. More importantly, regular tissue sourcing imparts variability that can make outcomes even less predictable. – AM

The most obvious is the ability to do autologous therapy, removing the need for immunosuppression and the risk of graft rejection. This includes the ability to genetically modify a patient-derived iPSC line, such as correcting disease-associated mutations. There are additional advantages, such as avoiding the ethical concerns of using hESC lines, and using a stem cell line derived from a fully consented individual. Also, iPSC lines can be derived using cGMP-compliant methods, whereas most hESC lines were not. – CM

iPS cells have a number of advantages over other cell therapy approaches. These advantages are the result of two fundamental biological properties of iPS cells; they replicate indefinitely (under tightly controlled culture conditions) and can be stimulated to differentiate into any cell type in the human body (if biological culture conditions are known). These two properties combine to make it an ideal cell therapy manufacturing platform. In addition, because iPS cells can be sourced from the peripheral blood of any individual, it gives us control over the genetic background of the source material. This allows either allogeneic or autologous cell therapy platforms. The science of stem cell manufacture has matured greatly over the past decade, making iPS cells more attractive as a manufacturing platform. And finally, the regulatory environment is becoming clearer, and the challenges are manageable. – EN

3) There is considerable interest in the ability to modify and edit iPSCs and design them to address a broad range of therapeutic needs. Can you describe this in more detail?

There are significant efforts ongoing in the field to try and generate engineered pluripotent stem cells for various applications including cell tracking, assay reporters, directed differentiation fate, delivery of therapeutic factors, etc. One current area of high interest within iPSC-based therapeutics is that where cells are being engineered as a means to render them compatible with all human immune systems. This is a technology that is still under development with work ongoing to show the system's full utility. Our current focus is on the use of iPSCs that have not been genetically engineered. – LC

There are many examples of this case but perhaps most notable are gene editing approaches that pertain to targeted immune therapies and as a mechanism to evade immune surveillance upon therapeutic introduction. In the first case, iPSCs can be engineered to express receptors targeted towards specific antigens (aka chimeric antigen receptor, CAR) by incorporating a modified gene cassette into the genome of the cell. These cells can then be differentiated into T cells that identify oncogenic signatures on the surface of cancer-associated cells and facilitate elimination of the offending cells that carry this signature. In the other example, researchers have manipulated the immunogenicity of a cell by eliminating the natural cell surface proteins responsible for inducing an immune response. Albeit yet to be proven, the rationale is to create a universal cell that will be unlikely to elicit an immune response upon therapeutic introduction because it does not carry the signatures identified by the body's immune system that result in a destructive response. – AM

Commentary: Therapeutic iPSCs

(Continued from Page 6)

A multitude of diseases result from the degeneration, dysfunction or death of cells. iPSCs, because they can become any type of cell, present an opportunity to address nearly all of these conditions by replacing the cells needed to perform critical functions. Combined with new gene editing technologies, the therapeutic possibilities of iPSCs are incredibly broad. In the future, editing may be used to introduce additional safety features (e.g. “suicide-switch”) or to increase efficacy (editing the cells to express additional therapeutic growth factors). – EN

4) In which disease areas are we likely to see the greatest early progress for iPSC therapeutics?

Based upon the current activity within the field it seems that retinal, neurodegenerative and cardiac iPSC-based therapeutics are three highly prioritized areas that may yield early progress. – LC

The first iPSC therapeutics will treat the same indications as ongoing hESC cell therapies: RPE for wet/dry AMD and Stargardt disease, midbrain DA neurons for PD, cardiomyocytes (cells and extracellular vesicles) for severe cardiac failure, beta cells for diabetes. In the long run, success for iPSC therapies will depend on a combination of efficacy and those cell types where immune rejection is a major hurdle. – CM

The indications where we are going to see the greatest progress are those where we have a clear understanding of the disease biology, as well as early indications that a cell therapy approach can address the pathology, for example, Parkinson’s disease. Clinical benefit has been seen in some patients with these conditions who have received cell therapies in early clinical trials. – EN

5) How much progress has been made in identifying and establishing HLA “super donor” cell lines that minimize immunogenicity concerns and enhance the versatility of the platform?

The establishment of HLA lines has gained international interest as other countries demonstrate progress towards banking in addition to the U.S., such as Sweden, Korea and Japan. The number of lines currently banked is relatively low due to donor recruitment and the expense incurred to support such regulated manufacture. As these banks continue to grow, standardization is a key topic under heavy discussion as it relates to characterization of iPSC banks. Depth of sequencing and method of analysis comprise a significant portion of these polarizing debates as it relates to disease-specific mutations. Deep sequencing is likely to unveil variants with unknown association to disease that could stymie progress while low-resolution sequencing may miss critical oncogenes. Therefore, standardizing bodies such as ISCBI (International Stem Cell Banking Initiative) and GAIT (Global Alliance for iPSC Therapies) are working hard to reach consensus on resolving these debates. – AM

A number of academic research groups and companies are working on creating super donor cell lines. These banks are in process, and depending on the definition of “matching,” could provide a beneficial match to a large percentage of the U.S. population. However, what would be ideal is a single cell line that is compatible with all potential patients and can be mass manufactured and available in an off-the-shelf manner, which is a significant focus of BlueRock’s platform development. – EN

6) What are some of the manufacturing advantages of iPSCs over other cell types? How is your organization approaching the issue of manufacturing strategy and scale-up?

The scalability of the biological starting material and the ability to genetic modify that material and comprehensively test it are among the advantages of using iPSCs over other cell types for manufacturing cell therapy products. In order to maintain manufacturing flexibility, we exclusively use single use disposables in both scale up and scale out manufacturing. We have focused on selecting and characterizing platform solutions for manufacturing operations across the multiple iPSC derived cell types in our portfolio. This allows us to leverage process understanding between projects to accelerate development. – PF

The advantage of iPSCs over other PSCs (e.g., ESCs) are the ability to source the PSC line from very specific donors (e.g., HLA homozygous individuals) in a manner that does raise any ethical or religious issues. CDI has utilized this strategy to address the main shortcoming of allogeneic cell therapies, rejection, by creating banks of iPSC lines from HLA homozygous donors thereby creating the ability to produce cell therapeutics with an HLA type that matches the intended recipient. The manufacturing and scale-up strategy will focus on establishing manufacturing processes that are robust enough to provide efficient differentiation of these HLA homozygous iPSC banks to the intended final cell product. Again, the ability to focus on optimizing the manufacturing process for this defined panel of cell banks represents a significant advantage over autologous approaches where wide patient-to-patient variability can result in additional significant manufacturing and quality challenges. – DH

From a manufacturing perspective, iPSCs can be made from any kind of cell and they can become any kind of cell, providing tremendous flexibility in sourcing for cell lines and ongoing manufacturing. Additionally, since iPSCs are derived from a known consenting donor with documented health history, you have the ability to control that donor profile for designability. Additionally, it provides the ability to follow up with that donor if needed to get additional information about their health, genetic profile, etc. BlueRock is establishing an R&D and cGMP manufacturing capabilities in Toronto. To facilitate and accelerate this buildout, we have forged partnerships with the University Health Network and the Centre for the Commercialization of Regenerative Medicine. – EN

899
Clinical trials underway
worldwide by mid-year 2017

Ph. I: 284

Ph. II: 539

Ph. III: 76

Number of Clinical Trials Utilizing Specific RM/AT Technology: Q2 2017

Gene Therapy & Gene-Modified Cell Therapy

Total: 504

Ph. I: 184

Ph. II: 286

Ph. III: 34

Cell Therapy

Total: 586

Ph. I: 174

Ph. II: 365

Ph. III: 47

Tissue Engineering

Total: 24

Ph. I: 6

Ph. II: 14

Ph. III: 4

- 447 (50%) of all current clinical trials are in oncology, including leukemia, lymphoma, and cancers of the brain, breast, bladder, cervix, colon and others.
- 89 (10%) are in cardiovascular disorders, including congestive heart failure, myocardial infarction, critical limb ischemia and others.
- 59 (6%) are in diseases of the central nervous system, including multiple sclerosis, Parkinson's disease, traumatic brain injury, ALS and others.

*Total number of clinical trials represents sector-wide figures; please note that products employing cell-based immunotherapy are accounted for in both the gene therapy & gene-modified cell therapy and cell therapy sectors. As a result, the total number of clinical trials does not equal the sum of the trials within the individual technology groups.

Commentary: Reimbursement & Market Access

Commentator: Mark Trusheim
Visiting Scientist, MIT Sloan School of Management
Strategic Director, NEWDIGS, MIT Center for Biomedical Innovation

1) Given the significant attention these days on drug pricing and cost, what are the unique challenges related to market access for disease-modifying and potentially curative gene and cell therapy products, some of which are autologous and will be administered as a hospital inpatient therapy?

The key, unique characteristic of durable or curative therapies is their one-time treatment cost linked to multi-year benefits. Most drugs are taken every day or month, therefore the treatment costs and the patient benefits are matched over time. Curative therapies disrupt this historic pricing-over-time approach.

A secondary unique characteristic is that, in many cases, we may not know exactly how long the therapy's effect will last. Will it be for three years, for ten years or for a lifetime? This adds uncertainty for everyone: patient, payer and innovative therapy developer. That uncertainty adds challenges to how to value and pay for these therapies. Time will tell, of course, but we don't want to prevent patient access until we have the results of a multi-decade clinical trial.

Another characteristic for many of these therapies is that many are for indications with very small patient populations. Many other 'orphan' therapies exist of course, but a large fraction of gene therapies in development are for orphan diseases.

2) As we know, the current health care system and payment models were not constructed with these types of advanced therapies in mind; now that several of these therapies are approaching near-term commercialization, what are the most pressing changes that must be made?

The current system doesn't work well for any of the participants (payers, developers, regulators, providers, patients and policy makers). These advanced therapies require that everyone change a little so that patients can win a lot. To be effective, those changes need to be coordinated rather than independent actions which might be oblivious to the impacts on the other stakeholders.

The most immediate challenge is not brainstorming changes, but creating the environment for the changes to be designed and coordinated outside of the pressure of a product access negotiation. The NEWDIGS consortium at MIT is one such multi-stakeholder group addressing these issues. The MIT NEWDIGS FoCUS (Financing and reimbursement of Cures in the U.S.) project provides a "think and do" platform for designing, evaluating and driving the timely implementation of nationally scalable solutions.

3) If a value-based, annuity or pay-for-performance type model is recommended, how can/should such a system be implemented? Can it be done gradually? Can pilot programs be utilized? How are different payer stakeholder groups likely to view these models?

A value- and performance-based annuity does provide a promising model to address many stakeholder concerns. It can smooth cash payments for payers while also addressing the efficacy and durability uncertainties of new therapies. Patients gain access and developers receive some revenues while all, including regulators and providers, benefit from continued learning about the therapy over time.

Whether implemented gradually or rapidly, annuity models require strong information systems to collect and evaluate the critical patient experience data to trigger the annuity payments.

4) With the unprecedented potential impact of these therapies, how can therapeutic developers address payer unfamiliarity and / or uncertainties?

Engaging with payers (and others) proactively during development is the key. Whether individually, and especially as a group through associations such as ARM, proactive engagement can help payers anticipate, appreciate and even accelerate these therapies.

5) In what way can therapeutic developers best demonstrate their products' value proposition to payers?

Nothing demonstrates confidence better than willingly approaching financial risk with payers based on the performance of the products. Of course, having strong clinical evidence of consistent, complete and durable efficacy eliminates most of the risk. However, not all conditions and therapies enable such strong, upfront data. A commitment to continued evidence collection, especially in real-world settings, is the next best thing, and it may be even better as it brings payers and developers together in helping provide the best outcomes for patients.

Examples of major milestones and key data events: Q2 2017

Cell-based immuno-oncology programs

Novartis Pivotal CTL019 6-Month Follow-Up Data Show Durable Remission Rates in Children, Young Adults with r/r B-cell ALL – June 23, 2017

Kite Reports 73 Percent of Patients Achieved MRD Negative Complete Remission in Updated Analysis From Phase 1 ZUMA-3 CAR-T Trial of KTE-C19 in Adult Patients With High Burden Relapsed/Refractory Acute Lymphoblastic Leukemia – June 5, 2017

Kite Receives U.S. Food and Drug Administration Priority Review for Axicabtagene Ciloleucel – May 26, 2017

FDA Grants Fast Track Designation for Celyad's Ischemic Heart Failure Therapy, C-Cure – May 11, 2017

Novartis CAR-T Cell Therapy CTL019 Receives FDA Breakthrough Therapy Designation for Treatment of Adult Patients with R/R DLBCL – April 18, 2017

Gene therapy programs

Abeona Therapeutics Receives FDA Orphan Drug Designation for ABO-201 Juvenile Batten Disease Gene Therapy Program – June 29, 2017

Fibrocell Receives Rare Pediatric Disease Designation from FDA for FCX-013 for Treatment of Localized Scleroderma – June 12, 2017

Sangamo Therapeutics And Pfizer Announce SB-525 Investigational Hemophilia A Gene Therapy Receives Orphan Medicinal Product Designation From The European Medicines Agency – June 7, 2017

Sangamo Receives Fast Track Designation From The FDA For SB-525 Investigational Hemophilia A Gene Therapy – May 18, 2017

Gene therapy programs (cont.)

- Spark Therapeutics Completes Rolling Biologics License Application Submission to FDA for Investigational Gene Therapy Voretigene Neparvovec – May 18, 2017
- Sangamo Therapeutics Announces Special Regulatory Designations from the FDA for Three Clinical Programs – May 4, 2017
- uniQure Receives European Medicines Agency Priority Medicines Designation for AMT-060 in Hemophilia B – April 25, 2017

Cell-based therapy programs

- Vericel Receives FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for Ixmyelocel-T for the Treatment of Advanced Heart Failure Due to Ischemic Dilated Cardiomyopathy – May 10, 2017
- jCyte Receives Regenerative Medicine Advanced Therapy Designation – May 2, 2017
- Kiadis Pharma Announces Filing of Marketing Authorization with the European Medicines Agency for ATIR101 in Blood Cancers – April 26, 2017
- Enzyvant Receives FDA Breakthrough Therapy Designation and Regenerative Medicine – April 17, 2017
- Mesoblast Announces Successful Interim Analysis in Phase 3 Heart Failure Trial – April 10, 2017



Current Regulatory & Legislative Priorities

Global reimbursement issues –

- Developing and advancing specific proposals and analyzing potential financing models to ensure a supportive reimbursement environment for gene and cellular therapies and other regenerative medicine products.
- Continued outreach to U.S. CMS, private payers and EU HTA bodies, reimbursement agencies and other stakeholders.
- Ensure any future proposals for ACA repeal / replace and Medicaid reform appropriately support reimbursement for regenerative medicine products and maintain patient access.

U.S. regulatory issues –

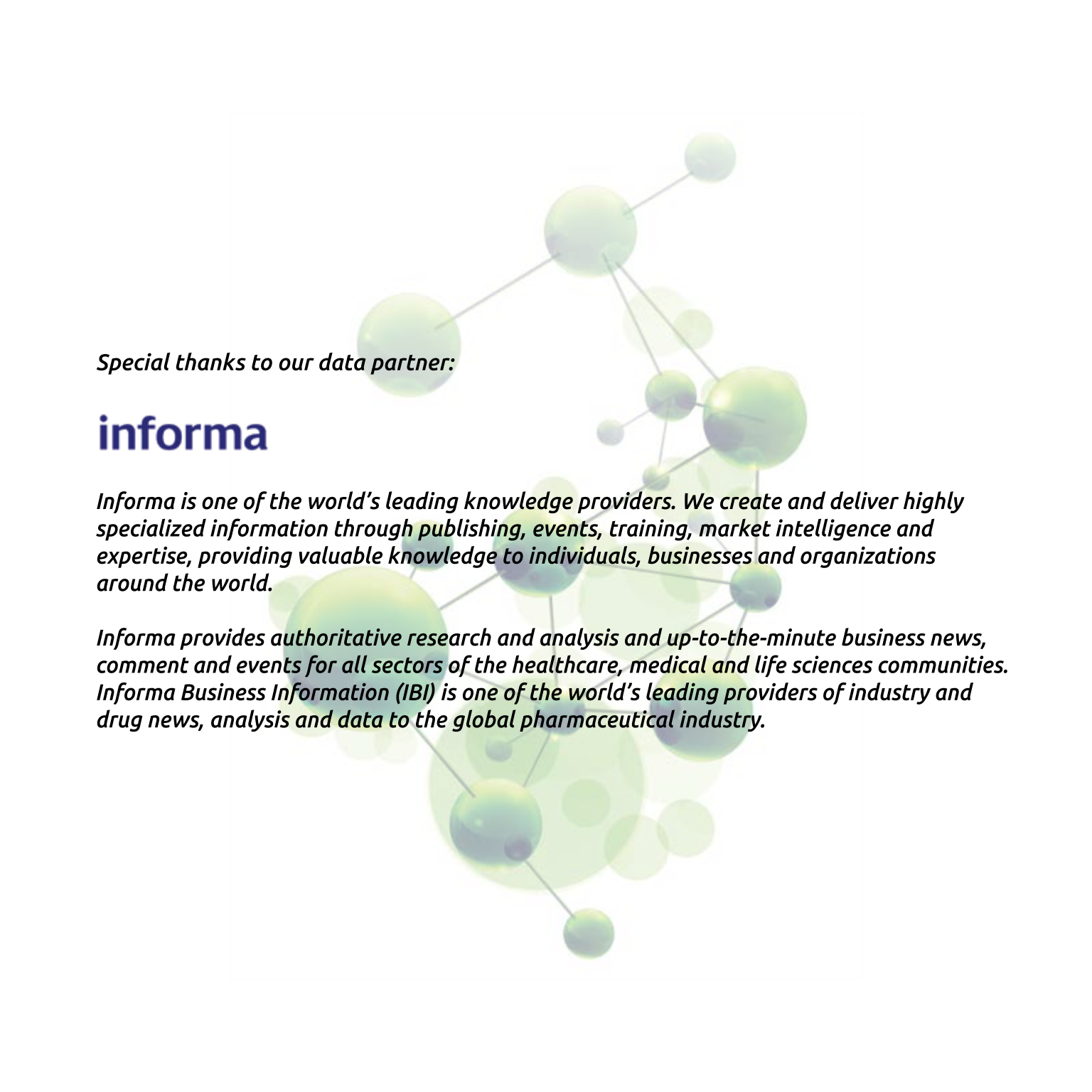
- Engaging with FDA on its implementation of the 21st Century Cures' new Regenerative Medicine Advanced Therapy designation, which optimizes a pathway to market for regenerative medicine products; ARM is working to ensure gene therapy products are eligible for the new designation.
- ARM is developing policy and advocacy approaches regarding expanded access to yet-unapproved products; ARM has expressed concerns with so-called “Right to Try” laws, especially in the context of cell and gene therapies and the potential safety impact on patients.
- ARM and the Regenerative Medicine Standards Coordinating Body (SCB), which was founded by ARM's Science & Technology Committee, are working with the FDA to validate and confirm the SCB as the best qualified organization to undertake the RMAT standards coordination role described in Section 3036 of the 21st Century Cures legislation.
- ARM supports a modified role for the NIH-Recombinant DNA Advisory Committee (RAC); ARM is identifying areas for improvement and ensuring the streamlined oversight of gene therapy clinical trials.

European regulatory issues –

- Promoting improvements of European regulatory procedures for clinical trials with advanced therapy medicinal products (ATMP) consisting of and/or containing genetically-modified organisms via ARM's soon-to-be-released position paper, which will be officially endorsed by a large coalition of trade associations.
- Evaluating EU legal frameworks on blood, tissues and cells via on-going public consultation, direct European Commission engagement and participation in an upcoming stakeholder meeting.
- Initiating a regional legal analysis regarding the issue of Hospital Exemption (HE), enabling EU ATMP developers to improve their understanding of the legal resources available to ensure fair implementation of HE across France, Germany, Italy, Spain and the UK.
- Defining and starting to deliver on a stakeholder engagement plan to educate and improve market access for ATMPs at the country level.

Genome editing & related bioethics issues –

- ARM continues to support and work closely with the National Academies of Sciences, Engineering and Medicine in their evaluation of the ethical, legal and safety issues connected to genome editing.
- ARM supports the Academies' additional international summits in 2018 (January in China; June in the U.K.) to determine the best path forward to monitor the advances in technology, scrutinize the ongoing and future worldwide research activity and to address the ethical, legal and critical safety issues.



Special thanks to our data partner:

informa

Informa is one of the world's leading knowledge providers. We create and deliver highly specialized information through publishing, events, training, market intelligence and expertise, providing valuable knowledge to individuals, businesses and organizations around the world.

Informa provides authoritative research and analysis and up-to-the-minute business news, comment and events for all sectors of the healthcare, medical and life sciences communities. Informa Business Information (IBI) is one of the world's leading providers of industry and drug news, analysis and data to the global pharmaceutical industry.



ALLIANCE_{for}
Regenerative Medicine

Alliance for Regenerative Medicine

Contact: Lyndsey Scull | Senior Director, Communications | Iscull@alliancerm.org

www.alliancerm.org