

Regenerative medicine: A growing global industry, ARM reports

By Peter Winter, *BioWorld Insight Editor*

SAN FRANCISCO – Last year at this time the regenerative medicine and advanced therapies industry had begun its coming-out party riding a wave of promising clinical data readouts that ignited strong investor interest and catalyzed involvement and interest from big pharma companies. This momentum has continued to increase during the past year, said Edward Lanphier, chair of the Alliance for Regenerative Medicine (ARM) at their state of the industry briefing delivered at the Biotech Showcase meeting here. In fact the sector, which comprises enterprises focused in the areas of cell therapy, tissue engineering and biomaterials and gene and gene-modified cell therapies and genome editing, has now evolved into a global industry, currently 500-plus companies strong, that continues to grow.

Lanphier presented the major highlights from a year that has seen a broad array of products in the pipeline grow substantially in 2014. ARM has identified just over 300 companies in the U.S. operating in the space with 157 in Europe and 53 in Asia. Encouragingly these

firms are involved in over 378 clinical trials and 65 percent of these have advanced to the phase II and phase III stages. In terms of fundraising 2014 was an exceptional year, noted Lanphier, with \$6.3 billion raised by regenerative medicine and advanced therapies companies representing a year-over-year growth of 112 percent. Approximately \$3 billion of this amount was raised by those companies involved in gene and gene-modified cell therapy compared with almost \$500 million in 2013.

The white-hot biotech initial public offering (IPO) market also embraced companies in the field that collectively raised \$1.3 billion. Among them was Juno Therapeutics Inc., of Seattle, which generated \$304 million from its IPO – making it the largest U.S. biotech IPO in the last 15 years. Juno's debut in December 2014 followed one day after

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Momentum building for regenerative medicine and gene therapy

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SAN FRANCISCO – The Washington-based Alliance for Regenerative Medicine (ARM) presented its state-of-the-industry briefing at the Biotech Showcase meeting last week, which re-affirmed the regenerative medicine and advanced therapies industry is extremely vibrant.

Last year's briefing reported that regenerative medicine had begun its coming-out party, riding a wave of promising clinical data readouts that ignited strong investor interest and catalyzed involvement and interest from big pharma companies. That momentum flowed over into 2014, according to Edward Lanphier, chair of the alliance and president and CEO of Sangamo Biosciences Inc.

This year, the alliance is representing an expanded base of companies thanks to an October 2014 merger with its European sister organization, the Alliance for Advanced Therapies (AAT) that served to create a global organization operating on behalf of the advanced therapies and regenerative medicine sector (cell therapy, gene and gene-modified cell therapies, genome editing, tissue engineering and biomaterials) in both the U.S. and Europe.

According to Lanphier, that global enterprise currently consists of

517 companies involved in the sectors they now represent, with 60 percent of the firms located in North America, 28 percent in Europe and just over 10 percent in Asia.

Encouragingly, those firms have so far brought 66 products to the marketplace and they collectively have a full pipeline with 378 ongoing clinical trials; 65 percent of clinical products have advanced to the phase II and phase III stages. As far as fundraising, 2014 was an exceptional year, noted Lanphier, with \$6.3 billion raised by regenerative medicine and advanced therapies companies, representing a year-over-year growth of 112 percent. Approximately \$3 billion of that amount was raised by those companies involved in gene and gene-modified cell therapy, compared with about \$500 million in 2013.

WHITE HOT

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fellow cellular immunotherapy firm Bellicum Pharmaceutical Inc. raised \$140 million.

Also contributing to the breakout year for the sector was a robust period of partnering activity. Among the many deals included U.K.-based Adaptimmune Ltd. that inked a \$350 million deal with Glaxosmithkline plc for its lead program NY-ESO-1, an

autologous T-cell product targeting the cancer testis antigen. As an encore, U.S. investors put \$104 million into the company to fund a significant expansion of their clinical development program.

Among ARM's 2015 advocacy aims include support of the enactment of the Regenerative Medicine Promotion Act and 21st Century Cures and the facilitation of expedited approval for regenerative medicine products and the promotion of a clear, predictable regulatory pathway.

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generated \$1.3 billion. Among them was Juno Therapeutics Inc., of Seattle, which garnered \$304 million from its IPO – making it the largest U.S. biotech IPO in the last 15 years. The company has clinical work ongoing with CAR T-cell therapies directed against CD19, which has reached the phase I/II stage. Juno's debut in December 2014 followed one day after fellow cellular immunotherapy firm Bellicum Pharmaceutical Inc. raised \$140 million.

Kite Pharma Inc. was another taking advantage of the immunoncology updraft, pricing an IPO for gross proceeds of \$146 million that will help advance its anti-CD19 CAR T-cell therapy.

Gene therapy technologies were also well represented by 2014 IPO graduates. Uniqure NV raised \$91.8 million from its IPO and Avalanche Biotechnologies Inc. received \$109 million from an upsized IPO.

Also contributing the breakout year for the sector was a robust period of partnering activity. Among the many deals included:

- Regeneron Pharmaceuticals Inc. signed an ophthalmology deal with Avalanche Biotechnologies Inc. that buys a time-limited right to first negotiations to phase II-stage AVA-101, targeting vascular endothelial growth factor for wet age-related macular degeneration. Regeneron will pay up to \$640 million in development and regulatory milestone rewards to Avalanche via an agreement that covers as many as eight therapeutic targets, with Regeneron keeping worldwide rights to each that is advanced into the clinic.
- U.K.-based Adaptimmune Ltd. inked a \$350 million deal

with Glaxosmithkline plc for its lead program NY-ESO-1, an autologous T-cell product targeting the cancer testis antigen. As an encore, U.S. investors put \$104 million into the company to fund a significant expansion of its clinical development program.

- Baxter International Inc. strengthened its own hemophilia franchise by snapping up the assets of Chatham Therapeutics LLC in a \$70 million deal plus potential development, regulatory and commercial milestone payments. The acquisition gives Baxter full access to Chatham's gene therapy platform, including a hemophilia B (FIX) program that was part of a 2012 collaboration between the companies to evaluate Baxter's BAX 335, a preclinical hemophilia A program and potential future application to additional hemophilia treatments.
- Cellectis SA and Pfizer Inc. formed a strategic collaboration in cancer based on the firm's allogeneic CAR T-cell platform, which will involve an initial outlay of about \$112 million, as well as research funding, milestones that could reach as much as \$2.775 billion in total, and tiered royalties on any products that reach the market.

Lanphier highlighted that among ARM's 2015 advocacy aims are the support of the enactment of the Regenerative Medicine Promotion Act that seeks to establish a national strategy to support regenerative medicines. Major provisions of the bill include creation of a multi-agency Regenerative Medicine Coordinating Council within the Department of Health & Human Services. It also calls for a detailed assessment of federal activities in regenerative medicine and their progress as compared to national programs in other countries.