

Cell Therapy 2015: With Maturity Comes Promise

The Life Sciences Report www.TheLifeSciencesReport.com

12/23/2014

COMPANIES MENTIONED

- Alliance for Regenerative Medicine
- Athersys Inc.
- Bavarian Nordic
- Cesca Therapeutics Inc.
- Dendreon Corp.
- Inovio Pharmaceuticals Inc.
- Mesoblast Ltd.
- NeoStem Inc.
- Opexa Therapeutics Inc.
- StemCells Inc.

Streetwise Reports LLC

101 Second St., Suite 110
Petaluma, CA 94952
Tel.: (707) 981-8107
Fax: (707) 981-8998

bfung@streetwisereports.com

THE ENERGY REPORT

THE GOLD REPORT

THE LIFE SCIENCES REPORT

THE MINING REPORT

A number of new and innovative medical paradigms are being explored by companies in the life sciences sector, with one of the front-runners being cell therapy—a disruptive technology that has, in the past few years, progressed out of preclinical studies and into clinical development. To learn more about the transformative promise of regenerative medicine, and what investors might expect from companies working in the sector in 2015, [The Life Sciences Report](#) asked analysts Jason Kolbert of Maxim Group and Dr. Christopher James of Brinson Patrick to discuss the advances that could generate robust returns on investment in coming years.

Source: [Tracy Salcedo-Chourré of The Life Sciences Report](#)

Cell Therapy in a Nutshell

Cell therapies have been gaining momentum in the life sciences for more than half a century. The first cell therapies, bone marrow transplants, were performed in the 1960s, and since then the idea of using healthy cells to replace or regenerate diseased cells has expanded into a number of indications, from cancer to cardiac disease to cosmetic therapy. Today, a number of companies are engaged in both clinical and preclinical trials, and there is some consensus that cell therapies will be "mainstream" in the near future.

The new year could prove a bellwether for regenerative medicine. The sheer volume of companies, universities and financial institutions conducting or backing research and development in the field attests to its potential: The [Alliance for Regenerative Medicine](#) (ARM), an advocacy group working with legislators, the investment community and regulatory agencies to promote development of cell therapy technologies, has more than 170 members working in the field.

On the horizon: Australian cell-therapy company [Mesoblast Ltd. \(MSB:ASE; MBLTY:OTCPK\)](#) is positioning itself for expedited approval of several of its allogeneic (derived from the same species) cell therapy products in Japan in 2015, following passage of legislation in that country that expedites approval. And though it hit a significant speed bump last month, [Dendreon Corp.'s \(DNDN:NASDAQ\)](#) Provenge (sipuleucel-T), an autologous (derived from and returned to the same patient) cell therapy for metastatic castrate-resistant prostate cancer, was the first FDA-approved cell therapy, and has helped pave a path forward for additional approvals in the U.S.

To get a handle on what investors can expect, and an idea of which companies to keep an eye on, [The Life Sciences Report](#) turned to Dr. Christopher James, managing director and senior equity research analyst with Brinson Patrick Securities Corp. and Jason Kolbert, managing director, senior biotechnology analyst and head of healthcare research with Maxim Group. Here's what they had to say about what 2015 might bring in both the sector and for certain companies working in the space.

Great Expectations

Asked which specific indications could be most responsive to cell therapies, and about companies working in those therapy areas, Kolbert provided several names.

The next "major event" in the sector will be results in a Phase 2 trial in ischemic stroke being conducted by [Athersys Inc. \(ATHX:NASDAQ\)](#), with results expected in Q1/2015, the analyst said. A number of other trials are also slated to start in 2015, including [NeoStem Inc.'s \(NBS:NASDAQ\)](#) Phase 3 trial in acute myocardial infarction and [Cesca Therapeutics Inc.'s \(KOOL:NASDAQ\)](#) pivotal trial in critical limb ischemia.

Kolbert is also looking forward to two trials involving Mesoblast's own stem cell product, as well as results from Prochymal (remestemcel-L, human mesenchymal stem cells for intravenous infusion, acquired from [Osiris Therapeutics Inc. \(OSIR:NASDAQ\)](#)). The Mesoblast product is expected to enter a pivotal trial in back pain, and the company is currently enrolling a global trial in congestive heart failure with partner [Teva Pharmaceutical Industries Ltd. \(TEVA:NASDAQ\)](#). The Osiris product is expected to be redeveloped in the U.S. for graft-versus-host disease (GvHD). Prochymal has been conditionally approved in Canada and New Zealand for GvHD in children. In addition, Mesoblast is expected to release data in a Phase 3 Prochymal trial in Crohn's disease.

James believes the area where cell therapy—cell regeneration—could have the biggest impact is also one of the riskiest—in the central nervous system (CNS), treating disorders or injury of the brain or spinal cord. James sees applications in multiple sclerosis (MS) and spinal cord injury, to regenerate neuronal function, and in Parkinson's disease, to address the death of dopaminergic cells. He also likes ocular applications, for treatment of such conditions as age-related macular degeneration (AMD).

James has two cell therapy companies under coverage. [Opexa Therapeutics Inc. \(OPXA:NASDAQ\)](#), with its Tcelna platform technology, is developing therapies in both CNS and the eye. A Phase 2 trial in secondary progressive MS is expected to read out in mid-2016. The company is also developing OPX-212, an autologous T-cell immunotherapy, to treat neuromyelitis optica, an autoimmune condition that leads to blindness.

The second company, [StemCells Inc. \(STEM:NASDAQ\)](#), is in a Phase 2 trial in the treatment of cervical spinal cord injury. On Dec. 18, the company announced that it had transplanted its HuCNS-SC (purified human neural stem cells) into its first patient; James expects final data from that trial in late 2015. Results from the company's Phase 1/2 trial in dry AMD, are expected in Q1/2015—perhaps as early as January.

StemCells' Phase 2 trial in cervical spinal cord injury points out what James sees as one of the regulatory hurdles cell therapies will have to overcome as they progress through the U.S. Food and Drug Administration's (FDA's) approval process. The trial is a randomized, controlled, *single-blind* study, as opposed to the FDA standard of double-blind pivotal studies.

To treat central nervous system disease or injury, companies must inject cells into a patient, a "surgical" intervention, James explained. But clinicians "can't do a sham surgery on the brain or on the spine. . .that's not ethical." So companies like StemCells must work with the FDA to develop pivotal trial designs that are "less stringent" than the double-blind model.

The Bigger Picture

Kolbert is enthusiastic about the prospects for cell therapies in 2015, and doesn't see much in the way of headwinds. "Cell therapy ultimately lowers the cost of treating disease, and at a time when there is an outcry over high-priced therapies, cell therapy offers promise to lower the cost of treating disease. In terms of politics, the cell therapies we are focusing on are typically not controversial (using adult stem cells, not cells from embryos). As such, we don't see any controversy. The big change in the landscape now is the acceptance that cell therapy is 'safe.'"

On the plus side of the spectrum is oncology. Using cell therapy in cancer immunotherapy "holds great promise," Kolbert continued. "The ability to rev up the immune system with checkpoint inhibitors, and couple that with therapeutics, like the therapies being developed by [ImmunoCellular Therapeutics Ltd. \(IMUC:OTCBB\)](#) and [Agenus Inc. \(AGEN:NASDAQ\)](#) targeting glioblastoma, and the work that [OncoSec Medical Inc. \(ONCS:OTCBB\)](#), [Inovio Pharmaceuticals Inc. \(INO:NYSE.MKT\)](#) and NeoStem are doing in melanoma, is exciting."

James and Kolbert agree that recent elections, in which Republicans gained control of both houses of Congress, shouldn't affect ongoing work in the cell therapy field. "They have bigger issues to deal with," James commented.

However, James believes cell therapy companies may suffer from an "overhang" following the recent bankruptcy of Dendreon Corp. And Kolbert cautions investors that second- and third-generation technologies always follow. Such is the case with [Bavarian Nordic's \(BAVA:OMX\)](#) Prostavac. Prostavac has the potential to be equivalent to, or even superior to, Provenge, but at a fraction of the cost. The Phase 3 Prostavac trial reports data next year.

Another concern for investors, according to James, is the question of whether cell therapy companies can manufacture their products on a commercial scale. But the analyst believes that concern can be addressed, and cites the StemCells model, in which, according to the company, "[c]ryopreserved lines of donor-derived cells can be reproduced at commercial scale as 'stem cells in a bottle,' then distributed for patient doses as needed, much like an off-the-shelf pharmaceutical product."

[Christopher James, M.D.](#), is a managing director and senior equity research analyst focusing on life sciences companies with strong growth potential and developing novel agents for serious diseases including cancer and infectious, neurological, inflammatory, metabolic and cardiovascular diseases. He was previously a senior equity research analyst at Rodman & Renshaw and MLV & Co. Prior to joining Brinson Patrick, Dr. James was chief medical officer and senior vice president of medical affairs at Retrophin, a biotechnology company focused on developing therapeutics for rare and devastating diseases. While at Retrophin, he played a pivotal role in the study design and subsequent acceptance of an investigational new drug application with the cardiorenal division of the FDA to initiate a Phase 2 clinical study in a rare kidney disease called focal segmental glomerulosclerosis. Dr. James has prior buy-side experience working at Trivium Capital Management and MSMB Capital Management. Dr. James trained in neurological surgery at Cornell-New York Hospital and Memorial Sloan Kettering Cancer Center. He obtained a medical degree from Yale University School of Medicine and a bachelor of science in biology from Cornell University.

[Jason Kolbert](#) has worked extensively in the healthcare sector as product manager for a leading pharmaceutical company, as a fund manager and as an equity analyst. Prior to joining Maxim Group, where he is head of healthcare

research, senior managing director and biotechnology analyst, Kolbert spent seven years at Susquehanna International Group, where he managed a healthcare fund and founded SIG's biotechnology team. Previously, Kolbert served as the healthcare strategist for Salomon Smith Barney. He is often quoted in the media and is a sought-out expert in the biotechnology field. Prior to beginning his Wall Street career, Kolbert served as a product manager for Schering-Plough in Osaka, Japan. He received a bachelor's degree in chemistry from State University of New York, New Paltz, and a master's degree in business administration from the University of New Haven.

Read what other experts are saying about:

- [Athersys Inc.](#)
- [NeoStem Inc.](#)
- [StemCells Inc.](#)

Want to read more *Life Sciences Report* interviews like this? [Sign up](#) for our free e-newsletter, and you'll learn when new articles have been published. To see recent interviews with industry analysts and commentators, visit our [Streetwise Interviews](#) page.

If you would like to comment on the content of this interview, engage with the interviewee or any of the companies discussed in this interview, or if you would like to be interviewed by *The Life Sciences Report*, please contact Brandon Fung of *The Life Sciences Report* at bfung@streetwisereports.com, (707) 981-8107.

[The Gold Report](#) : [The Energy Report](#) : [The Mining Report](#) : [The Life Sciences Report](#)

IMPORTANT DISCLOSURES

- 1) Tracy Salcedo-Chourre compiled this article for Streetwise Reports LLC, publisher of *The Gold Report*, *The Energy Report*, *The Life Sciences Report* and *The Mining Report*, and provides services to Streetwise Reports as an employee. She owns, or her family owns, shares of the following companies mentioned in this interview: None.
- 2) The following companies mentioned in the interview are sponsors of Streetwise Reports: NeoStem Inc., Athersys Inc., StemCells Inc., Inovio Pharmaceuticals Inc. Mesoblast Ltd. is not affiliated with Streetwise Reports. The companies mentioned in this interview were not involved in any aspect of the interview preparation or post-interview editing so the expert could speak independently about the sector. Streetwise Reports does not accept stock in exchange for its services.
- 3) Christopher James: I own, or my family owns, shares of the following companies mentioned in this interview: None. I personally am, or my family is, paid by the following companies mentioned in this interview: None. My company has a financial relationship with the following companies mentioned in this interview: Opexa Therapeutics. I was not paid by Streetwise Reports for participating in this interview. Comments and opinions expressed are my own comments and opinions. I determined and had final say over which companies would be included in the interview based on my research, understanding of the sector and interview theme. I had the opportunity to review the interview for accuracy as of the date of the interview and am responsible for the content of the interview.
- 4) Jason Kolbert: I own, or my family owns, shares of the following companies mentioned in this interview: None. I personally am, or my family is, paid by the following companies mentioned in this interview: None. My company has a financial relationship with the following companies mentioned in this interview: None. I was not paid by Streetwise Reports for participating in this interview. Comments and opinions expressed are my own comments and opinions. I determined and had final say over which companies would be included in the interview based on my research, understanding of the sector and interview theme. I had the opportunity to review the interview for accuracy as of the date of the interview and am responsible for the content of the interview.
- 4) Interviews are edited for clarity. Streetwise Reports does not make editorial comments or change experts' statements without their consent.
- 5) The interview does not constitute investment advice. Each reader is encouraged to consult with his or her individual financial professional and any action a reader takes as a result of information presented here is his or her own responsibility. By opening this page, each reader accepts and agrees to Streetwise Reports' terms of use and full legal [disclaimer](#).
- 6) From time to time, Streetwise Reports LLC and its directors, officers, employees or members of their families, as well as persons interviewed for articles and interviews on the site, may have a long or short position in securities mentioned. Directors, officers, employees or members of their

families are prohibited from making purchases and/or sales of those securities in the open market or otherwise during the up-to-four-week interval from the time of the interview until after it publishes.

The Gold Report, *The Energy Report* and *The Life Sciences Report* do not render general or specific investment advice and do not endorse or recommend the business, products, services or securities of any industry or company mentioned in this report.

From time to time, Streetwise Reports LLC and its directors, officers, employees or members of their families, as well as persons interviewed for articles on the site, may have a long or short position in securities mentioned and may make purchases and/or sales of those securities in the open market or otherwise.

Streetwise Reports LLC does not guarantee the accuracy or thoroughness of the information reported.

Streetwise Reports LLC receives a fee from companies that are listed on the home page in the "Learn More About Companies in this Issue" section. Their sponsor pages may be considered advertising for the purposes of 18 U.S.C. 1734.

OTHER DISCLOSURES

Streetwise - *The Gold Report*, *The Energy Report* and *The Life Sciences Report* are Copyright © 2014 by Streetwise Reports LLC. All rights are reserved. Streetwise Reports LLC hereby grants an unrestricted license to use or disseminate this copyrighted material (i) only in whole (and always including this disclaimer), but (ii) never in part.