

WORLD

# StemCells

Regenerative Medicine Congress 2013

## 8 Exciting Stem Cell- Based Therapies in Late Stage Development



## Innovative breakthrough cell therapies in Phase III development

The cell therapy industry continued to grow in 2012, and as the big pharma and wider investor community increasingly turn their attention towards highly innovative stem cell-based therapies, 2013 is set to be a pivotal year for the future success of regenerative medicine.

Industry-sponsored clinical trials continue to progress well with approximately 50 late stage studies presently ongoing, many of which are poised to gain approval and reach patients in the coming 12 months. Therefore, as part of our eBook series in the run up to the World Stem Cells & Regenerative Medicine Congress in London, we have profiled 8 exciting therapies currently in phase III clinical development that promise to transform the lives of patients in the near future.

### Comments are always welcome so let us know your thoughts on these follow up points:

Which one of the therapies will be the first to gain regulatory approval?

What other late-stage therapies need to be added to this list?

What are the commercialisation challenges that lie ahead for these therapies when they successfully receive market authorisation?

I hope you enjoy the piece and make sure you share this with your colleagues.



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# Shire Regenerative Medicine

On the 8<sup>th</sup> February 2013, Shire Regenerative Medicine announced the initiation of their phase III study of ABH001 for the treatment of non-healing wounds in patients with Epidermolysis Bullosa.

[ClinicalTrials.gov link >](#)

## **Taken from the official press release:**

ABH001 for EB has been granted an orphan drug designation in the US and EU, and has also received Fast Track designation from the US Food and Drug Administration (FDA), which is aimed at facilitating the development and expediting the review of drugs and biologics that fill an unmet medical need. In addition, the European Medicines Agency's Pediatric Committee has agreed on a pediatric investigation plan for ABH001 for the treatment of EB.

The new Phase 3 study is a multi-site, prospective, randomized, open-label, intra-subject controlled trial evaluating the efficacy and safety of ABH001 to initiate healing and reduce the wound surface area of selected stalled, chronic cutaneous wounds associated with generalized EB. Approximately 20 subjects with generalized EB aged three years and older are planned to enroll in the trial, which is targeted to be conducted in 10 to 15 sites across the US, Europe and Canada. The study will comprise ABH001 applications sufficient to cover the surface area of the wound, applied topically every 4 weeks with protocol-specified dressings until healed or for up to 24 weeks.

"We are excited that Shire Regenerative Medicine has launched this trial," said Brett Kopelan, Executive Director of the Dystrophic EB Research Association of America (DebRA ) and father to a 5-year-old girl with recessive dystrophic EB. "While there is currently no cure for EB, I am encouraged that ABH001 is...targeting the chronic wounds that are the hallmark of this disease. I applaud Shire for pushing this forward and look forward to working closely with them as the trial progresses."



*"We are very eager to begin evaluating ABH001 as a potential wound treatment option for people with EB. We believe it has the potential to initiate and continue wound healing in this patient population," said Jeff Jonas, MD, President of Shire Regenerative Medicine.*

*"We are committed to developing regenerative medicine solutions that enable people with life-altering conditions to lead better lives, and are encouraged by the fast track and orphan drug designations we have received to further develop this potential therapy for people, most often young children, suffering from this devastating condition."*

# Aastrom Bioscience

Aastrom Biosciences have an ongoing pivotal Phase 3 clinical trial (REVIVE) for patients with critical limb ischemia and who have no option for revascularization.

[ClinicalTrials.gov link >](#)

## **Taken from the official press release:**

The Phase 3 trial, called REVIVE, is taking place around the country, including locally through the Michigan CardioVascular Institute. Critical limb ischemia is a form of peripheral arterial disease that occurs when arteries supplying blood to the legs become blocked. This can cause pain or numbness in the feet, open sores or ulcers and eventually gangrene of the legs or feet.

The condition affects about 1 to 1.5 million Americans overall. A good number of them can be helped with procedures to bypass the arteries, but about 25 to 30 percent of patients cannot have that kind of treatment, according to Dr. Safwan Kassas, director of the Cardiovascular Cell Therapy and Regenerative Medicine Program at Michigan CardioVascular Institute.

“Out of this 25-30 percent, today, there is no treatment,” he said. “The FDA has not approved any treatment for them. They’re really in a very dismal spot.” Statistics show within first year of developing critical limb ischemia, 25 percent of people will die, 25 percent will have amputation and 50 percent will be alive without amputation, Kassas said. He said in the second year, the chances of amputation or death increase.

“That created the challenge for the scientific community and medical community that something needed to be done,” he said. “That’s where stem cell treatment today is leading the way for treatments.”

[\*\*Full press release here >\*\*](#)



**Dan Orlando**, Chief Commercialisation Officer, **Aastrom Biosciences** and previously VP Business Development for North & South America at Takeda Pharmaceuticals will be presenting at the World Stem Cells & Regenerative Medicine Congress in London on **“How to get a larger partner bought into your innovation”**.

# Osiris Therapeutics

Osiris Therapeutics currently has a phase III trial for several indications including Crohn's Disease and acute graft versus host disease (GvHD).

[ClinicalTrials.gov link >](#)

[ClinicalTrials.gov link >](#)

## **FDA Fast-Track clearance expedites stem cell therapy**

Osiris Therapeutics, Inc. currently has two product candidates in clinical trials for a number of indications. Prochymal, an intravenously administered formulation of mesenchymal stem cells, is being evaluated in Phase 3 clinical trials for several indications, including acute graft versus host disease (GvHD) and also Crohn's disease. It is the only stem cell therapeutic currently designated by FDA as both an Orphan Drug and Fast Track product.

Prochymal is also being evaluated in Phase 2 clinical trials for the repair of heart tissue following a heart attack, the protection of pancreatic islet cells in patients with type 1 diabetes, and the repair of lung tissue in patients with chronic obstructive pulmonary disease.



*“Keen to learn more successfully commercialising cell therapies? Take a look at the PRESENTATION delivered by David Smith, Global Head Therapeutic Cell Solutions, Lonza at last year’s World Stem Cells & Regenerative Medicine Congress.”*

# Baxter

Baxter International have initiated a Phase III Autologous Stem Cell Clinical Trial to Improve Patients with CMI

[ClinicalTrials.gov link >](#)

## **Taken from the StemSave blog:**

Baxter International Inc. has initiated a phase III pivotal clinical trial to evaluate the use of adult autologous (an individual's own) stem cells to increase exercise capacity in patients who suffer from chronic myocardial ischemia (CMI), a disease that results in reduced blood supply to the heart that can lead to long-term tissue damage and heart failure.

"The prospect of using a person's own adult stem cells direct lender payday loans to restore and repair blood flow in CMI is a very exciting concept based on a biological regenerative approach," said Norbert Riedel, Ph.D., Baxter's chief science and innovation officer. "The goals of this phase III trial are aligned with Baxter's overall mission to develop life-saving and life-sustaining therapies and it will help us determine if the therapy can make a meaningful difference for CMI patients."

Baxter is one of many companies looking towards stem cell research as one of the most viable options in treating genetic and degenerative disease. As stem cell therapies such as these continue to revolutionize the field of medicine, preserving one's own stem cells becomes an important part of ensuring future access to cutting edge medical care.

The Baxter logo is displayed in white, italicized, sans-serif font against a dark blue rectangular background.

Want to discover a  
stock market analyst  
perspective of the  
biotechnology and  
regenerative  
medicine in 2013,  
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# Cardio3 Biosciences



At the end of 2012, Cardio 3 Bioscience gained authorisation to Begin World's First Phase III Clinical Trial in Regenerative Medicine for Heart Failure

[ClinicalTrials.gov link >](http://ClinicalTrials.gov)

## Taken from a press release:

The Belgian biotechnology company, Cardio3 BioSciences (C3BS), a leader in the discovery and development of regenerative and protective therapies for the treatment of cardiac diseases, today announces it has received authorization from the Belgian Federal Agency for Medicines and Health Products (FAMHP) to begin its Congestive Heart failure Cardiopoietic Regenerative Therapy (CHART-1) European Phase III trial for C3BS-CQR-1 in Belgium. This represents a world premiere for a regenerative medicine product targeting heart failure to be tested in the context of a Phase III trial. C3BS-CQR-1 is an autologous stem cell therapy for heart failure.

The Phase III trial is a prospective, multi-centre, randomized, sham-controlled, patient-and evaluator-blinded study comparing treatment with C3BS-CQR-1 to a sham treatment. The trial will recruit a minimum of 240 patients with chronic advanced symptomatic heart failure. The primary endpoint of the trial is a composite endpoint including mortality, morbidity, quality of life, Six Minute Walk Test and left ventricular structure and function at 9 months post-procedure. Studies in additional countries will commence once national regulatory approvals have been received.

The Cardio3 BioSciences therapy, called C3BS-CQR-1, involves taking stem cells from a patient's own bone marrow and through a proprietary process called Cardiopoiesis, re-programming those cells so that they go onto becoming heart cells. The cells, known as cardiopoietic cells, are then injected back into the patient's heart through a minimally invasive procedure using a catheter called C-Cath<sub>ez</sub><sup>®</sup>, with the aim of repairing damaged tissue and improving heart function and patient clinical outcomes. C3BS-CQR-1 is the outcome of multiple years of research conducted at Mayo Clinic (Rochester, Minnesota, USA), Cardio3 BioSciences (Mont-Saint-Guibert, Belgium) and Cardiovascular Centre in Aalst (Aalst, Belgium). This Phase III trial builds on the successful outcome of the Phase II trial conducted between 2009 and 2010 in multiple clinical sites in Belgium, Serbia and Switzerland.

# Gamida Cell

Earlier this year, Gamida Cell's StemEx®, involved in the treatment of patients with hematological malignancies such as leukemia and lymphoma, achieved its Primary Endpoint in Phase II/III Clinical Study.

[ClinicalTrials.gov link >](#)



## Taken from the official press release:

The primary endpoint is defined as the rate of mortality (%) within 100 days after transplantation. For the final analysis, the historical control cohort was comprised of a similar group of patients each transplanted with double cord blood during the years 2006-2010. The analysis shows 15.8% mortality in the StemEx group and 24.5% in the control group ( $p=0.034$ ). A complete analysis of the data will be available in a few weeks.

Twenty-five bone marrow transplantation centers worldwide treating 101 patients with hematologic malignancies following myeloablative therapy who could not find a family related matched bone marrow donor participated in the study.

"We are delighted with today's results of the Phase II/III study of StemEx. We remain focused on completing the development of StemEx as an alternative stem/progenitor cell source for transplantation in patients who cannot find a family related matched bone marrow donor," said Dr. Yael Margolin, president and CEO of Gamida Cell.

StemEx is a graft of an expanded population of stem/progenitor cells, derived from part of a single unit of umbilical cord blood and transplanted by IV administration along with the remaining, non-manipulated cells from the same unit. Cord blood has less matching requirements than bone marrow or peripheral blood transplants, providing the potential to increase the number of suitable transplant matches and to shorten the time it can take to find a match. However, there are a limited number of stem/progenitor cells in cord blood, enabling a quantity sufficient generally only for pediatric treatment. StemEx employs a technology that expands this small number of cord blood stem/progenitor cells, increasing their therapeutic capacity for transplantation in adolescents and adults

Download the full  
press release to  
learn more  
about Gamida  
Cell's StemEx  
pivotal trial



# PharmiCell



PharmiCell is currently planning a Multi-center, Open-label, Comparison and a Parallel Group Study (3 Groups) Phase 3 Clinical Trial for a Comparative Evaluation With the Existing Treatments, in Order to Verify the Long-term Efficacy and Safety of the First Cell Treatment Using Hearticellgram-AMI(Autologous Human Bone Marrow Derived Mesenchymal Stem Cells) in AMI Patients, and to Observe the Efficacy of the Second Cell Treatment.

[ClinicalTrials.gov link >](#)



[Click on the video above to find out more >](#)

# Tigenix

In 2012, Tigenix enrolled their first patient in the ADMIRE-CD trial, its pivotal Phase III clinical trial with Cx601, an adipose derived allogeneic stem cell suspension for the treatment of complex perianal fistulas in Crohn's disease patients.

## [ClinicalTrials.gov link >](#)

This multicenter, randomized, double-blind, placebo-controlled Phase III trial will enroll approximately 278 patients at 46 centers in 7 European countries and Israel. The main objectives of the study are to demonstrate safety and superior efficacy over placebo in perianal fistulas in Crohn's disease patients who failed to respond to previous treatment(s), in most cases biologicals, and to confirm the strong safety and efficacy results from the Phase II trial completed in 2011. Final results of the trial are expected in H2 2014, and, if positive, allow the Company to file for marketing authorization with the European Medicines Agency.

In phase II, Cx601 demonstrated a very high efficacy level (56%) compared to other products in the closing of fistulas in Crohn's patients. The trial also confirmed the strong safety profile of the product. Cx601 acts by controlled reduction of inflammation in the fistula, promoting adequate homeostasis through the release of anti-inflammatory factors which in turn promote natural fistula closure.



TIGENIX  
Living Medicines

You can freely  
**download**  
Eduardo Bravo, CEO,  
Tigenix's  
presentation from  
last year's World  
Stem Cells &  
Regenerative  
Medicine Congress –  
"Progressing into  
Phase III with an  
orphan indication"

# Want to know more...

This is the second in the series of eBooks designed to provide insight and expertise from a selection of the 60+ speaker faculty confirmed to lead the conversation at the 8<sup>th</sup> Annual World Stem Cells & Regenerative Medicine Congress 2013. So if you see value in learning more from these pioneering companies, there may be plenty more reasons to attend:

- Could you further streamline your clinical development to minimise lag between approval and reimbursement coverage?
- Have you identified your therapy's future manufacturing challenges and created a flexible, scalable and economic commercialisation program?
- Have you mapped out the commercialisation strategy for your cell therapy pipeline?
- Do you know how to exploit alternative methods and non-traditional sources of financing?
- Have you investigated the health economics to understand the reimbursement landscape market penetration of your cell therapy?
- Do you need expertise to balance resource constraints and regulatory requirements within your clinical development plan?
- Are you up to date with the therapeutic potential of iPS cells and discover cutting edge applications of stem cells in drug discovery?



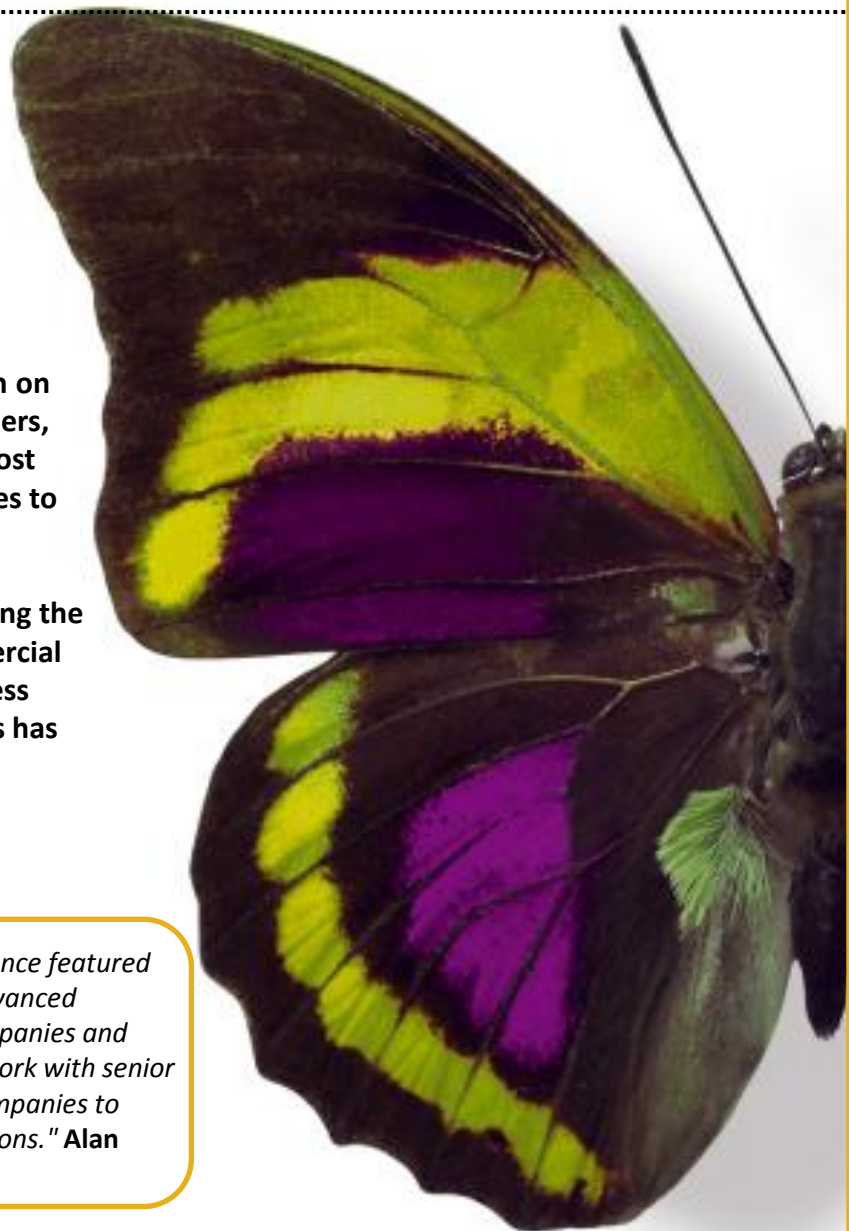
# We'd love to meet you...

The World Stem Cells & Regenerative Medicine Congress Europe in London on the 21<sup>st</sup> -23<sup>rd</sup> May 2013, brings together the late stage cell therapy developers, key opinion leaders, senior regenerative medicine stakeholders and the most innovative stem cell researchers to share expertise and discuss what it takes to achieve successful clinical development and commercialise to scale.

The meeting has evolved to cater for the challenges and opportunities facing the maturing stem cells & regenerative medicine industry. As Europe's commercial marketplace to engage, network and debate with the contacts your business needs to succeed, the World Stem Cells & Regenerative Medicine Congress has established itself as one of the premier meeting points where lasting collaborations and industry shaping conversations take place.

*"Commercial focus & speakers from industry business leaders were the major draws for me, and the content lived up to my expectations. Top-class execution by Terrapinn."* **Vincent Guercio**, Director, Cell Therapy, **Pall Corporation**

*"This well organized conference featured the progress of the most advanced regenerative medicines companies and allowed ample time to network with senior executives in all of these companies to initiate potential collaborations."* **Alan Lewis**, CEO, **Medistem**



# We'd love to hear your views on all of this...

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