

Chair Introduction

Welcome to the 5th Informa Cell Therapy Manufacturing Conference

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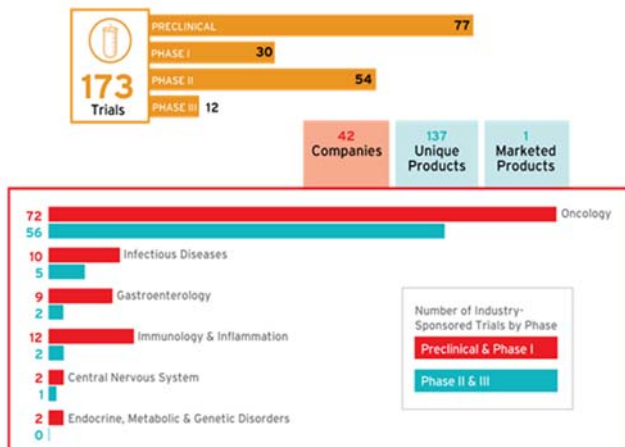
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Consulting on Advanced Biologicals

Immunotherapies are HOT

Cell-Based Immunotherapy Companies



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Source: Alliance for Regenerative Medicine

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Janssen Goes for Allo



Transposagen

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Transposagen Biopharmaceuticals Enters into Research Collaboration and Worldwide License Agreement with Janssen for Allogeneic Chimeric Antigen Receptor (CAR) T-Cell Therapies

Monday, November 24, 2014 12:03:52 PM America/New_York

November 24, 2014 – Transposagen Biopharmaceuticals, Inc., (Transposagen) a private biotechnology company headquartered in Lexington, KY, today announced they have entered into a research collaboration and worldwide license agreement with Janssen Biotech, Inc. (Janssen) to develop allogeneic Chimeric Antigen Receptor T-cells (CAR-T). To date, CAR-T therapies have shown promise in early human clinical trials for the treatment of blood cancers and allogeneic CAR-Ts have the potential for use as off-the-shelf cancer treatments without the need of matching donor with recipient.

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Consulting on Advanced Biologicals

FDA Approvals – Post 2001

Product	INN/Description	Type	Indication/s	Company	Type	Approved
Provenge	Sipuleucel-T	Autologous - Immunotherapy	asymptomatic or minimally symptomatic metastatic castrate resistant (hormone refractory) prostate cancer.	Dendreon Corporation	BLA	Apr 10
LaViv	Azficel-T	Autologous - Skin rejuvenation	For the improvement of the appearance of moderate to severe nasolabial fold wrinkles in adults.	Fibrocell Technologies, Inc.	BLA	Jun 11
Hemacord	Hematopoietic Progenitor Cells, Cord	Allogeneic - Cord blood	unrelated donor hematopoietic progenitor cell transplantation	New York Blood Center	BLA	Nov 11
Gintuit	Allogeneic Cultured Keratinocytes and Fibroblasts in Bovine Collagen	Allogeneic - Engineered Skin	treatment of mucogingival conditions in adults	Organogenesis	BLA	Mar 12
DuCord	Hematopoietic Progenitor Cells, Cord	Allogeneic - Cord blood	unrelated donor hematopoietic progenitor cell transplantation	Duke University	BLA	Apr 12
HPC, Cord Blood	Hematopoietic Progenitor Cells, Cord	Allogeneic - Cord blood	unrelated donor hematopoietic progenitor cell transplantation	Clinimmune Labs	BLA	May 12
AlloCord	HPC, Cord Blood	Allogeneic - Cord blood	unrelated donor hematopoietic progenitor cell transplantation	SSM Cardinal Glennon Children's Medical Center	BLA	May 13
None	HPC, Cord Blood	Allogeneic - Cord blood	unrelated donor hematopoietic progenitor cell transplantation	LifeSouth Community Blood Centers, Inc	BLA	Jun 13

EMA Submissions and Approvals

Last Year's Tally

Product	INN/description	Indication/s (Therapeutic area)	Company	Submission Date	Opinion	Finalisation Date
<i>Apligraf</i>	<i>Graftskin</i>	<i>Venous leg ulcers</i>	<i>Novartis (Licensing deal with Organogenesis)</i>	<i>Apr 2001</i>	<i>Unknown</i>	<i>Withdrawn 2002</i>
ChondroCelect	characterised viable autologous cartilage cells expanded ex vivo expressing specific marker proteins	cartilage defects of the femoral condyle of the knee	Tigenix	Jun 2007	Positive	Jun 2009
<i>OraNera</i>	<i>Autologous oral mucosal epithelial cells</i>	<i>(Ophthalmologicals)</i>	<i>CellSeed Europe Ltd</i>	<i>Jun 2011</i>	<i>(Negative)</i>	<i>Withdrawn Mar 2013</i>
<i>Hyalograft C</i>	<i>Cultured autologous chondrocytes on hyaluronan based scaffold</i>	<i>(Other medicines for disorders of the musculo-skeletal system)</i>	<i>Anika Therapeutics</i>	<i>Feb 2011</i>	<i>(Negative)</i>	<i>Withdrawn Jan 2013</i>
Provenge	Sipuleucel-T; autologous peripheral blood mononuclear cells activated with PAP-GM-CSF	(Immunostimulants)	Dendreon	Feb 2011	Positive	Jun 2013
MACI	Autologous cultured chondrocytes	(Other medicines for disorders of the musculo-skeletal system)	Genzyme Europe B.V.	Sep 2011	Positive	Apr 2013
Undisclosed	Spheroids of human autologous matrix associated chondrocytes	(Other medicines for disorders of the musculoskeletal system)	Undisclosed (assumed co.don AG)	Jan 2013	TBD	TBD
Holoclar	Ex vivo autologous corneal epithelial cells including stem cells	Ophthalmologicals	Chiesi Farmaceutici S.p.A.	Apr 2013	TBD	TBD

Dendreon Struggling

Dendreon Cuts 150 Jobs, As Prostate Cancer Drug Sales Decline

Luke Timmerman | 11/12/13 | [Follow @ldtimmerman](#)

Dendreon once had 2,000 employees at its peak, less than three years ago. Its stock was once over \$40 a share. Analysts once predicted its prostate cancer drug would rake in billions in sales every year, with no generic competition in sight.

Today, it's down to 820 employees, its stock is under \$3, and it's doing another mass layoff just to hold onto its diminishing cash reserves.



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Automation they key?

Argos Aims To Automate Cell-Based Cancer Therapy

Posted by Gabrielle on Nov 20, 2014

DURHAM, N.C. – Eventually, someone is going to have clinical and commercial success with a personalized cancer therapy. Learning from the mistakes of Dendreon and others, Argos expects to be first. Automation is its key.

- Argos Therapeutics has developed a cell-based therapy to help the body's own immune system fight cancer already present, on a tumor-specific and patient-specific basis.
- The Arcelis platform technology can, in theory, boost immune response to many kinds of solid tumors. Argos aims to prove its worth, first as an add-on to standard-of-care treatment for advanced kidney cancer.
- Final Phase III clinical data should come in mid-2016. Preparing to launch product as soon as regulators say OK, Argos began automating its manufacturing process a decade ago.
- Intent on commercial as well as clinical success, Argos has applied lessons learned by others including the ill-fated cell-therapy pioneer Dendreon. Everyone in the field must confront similar challenges.

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Dendreon Chapter 11

9th Nov 2014

The Seattle Times
Winner of Nine Pulitzer Prizes

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Originally published November 9, 2014 at 11:22 PM | Page modified November 11, 2014 at 9:20 AM

Biotech Dendreon files for Chapter 11 to restructure massive debts

Dendreon said late Sunday it has filed for bankruptcy protection as part of a plan to restructure \$620 million in debt, a move likely to effectively wipe out the value of its common stock.

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By Seattle Times business staff

Dendreon said it has filed for bankruptcy protection as part of a plan to restructure \$620 million in debt, a move likely to effectively wipe out the value of its common stock.

The biotechnology company, once Seattle's largest, filed a voluntary Chapter 11 bankruptcy petition Monday in Delaware.

Dendreon, which pioneered a therapy for treating prostate cancer with boosted cells from a patients own immune system, said the plan should allow continued operations and the ongoing commercial



DENDREON

Advanced Biologicals

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Who Wants This?

GEN News Highlights

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Apr 21, 2014

Aastrom Nabs Sanofi's Cell Therapy Business for \$6.5M

Aastrom Biosciences is picking up **Sanofi's Cell Therapy** and **Regenerative Medicine** (CTRM) business for \$6.5 million, gaining global commercial rights to three autologous cell therapy products:

- Carticel® (autologous cultured chondrocytes), an autologous chondrocyte implant (ACI) marketed in the U.S. for the treatment of articular cartilage defects;
- Epicel® (cultured epidermal autografts), a permanent skin replacement for full thickness burns greater than or equal to 30% of total body surface area, marketed worldwide; and
- MACI® (matrix-induced autologous chondrocyte implant), a third-generation ACI product marketed in the EU.

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Provenge	Sipuleucel-T; autologous peripheral blood mononuclear cells activated with PAP-GM-CSF	Company in Chapter 11			Positive	Jun 2013
MACI	EU Manufacturing Site Closed		Genzyme Europe (2011)	Apr 2011	Positive	Apr 2013
Undisclosed	Spheroids of human autologous matrix associated chondrocytes	(Other medicines for disorders of the musculoskeletal system)	Undisclosed (assumed co.don AG)	Jan 2013	TBD	TBD
Holoclar	Ex vivo autologous corneal epithelial cells including stem cells	Ophthalmologicals	Chiesi Farmaceutici S.p.A.	Apr 2013	TBD	TBD
Heparesec	Allogenic human heterologous liver cells	Allogenic human heterologous liver cells	Cytonet GmbH&Co KG	Feb 2014	TBD	TBD
Zalmoxis	Allogeneic T cells genetically modified to express suicide gene	Antineoplastic medicines	MolMed SpA	Apr 2014	TBD	TBD

A Reminder Change is Inevitable#1

ATMP major variations as of Nov 2014

- By 2013 there were 4 approved ATMP, 3 cell and 1 gene.
- Note: Type II variations are typically significant manufacturing changes that require some sort of comparability data.
- **Type-II variation Fee €3,600**

Variations (Type II) for authorised ATMP

	2009	2010	2011	2012	2013	2014	Total
Positive draft Opinion	0	0	1	1	9	4	15

A Reminder Change is Inevitable#2

ChondroCelect

II/0001	<p>Changes to the manufacturing process of the active substance.</p> <p>B.I.d.1.b.2 - Stability of AS - Change in the storage conditions - Change in storage conditions of biological/immunological ASs, when the stability studies have not been performed in accordance with a</p>	17/02/2011	02/03/2011
II/0006	<p>to add a new manufacturing site for ChondroCelect drug product and to submit results from user testing of package leaflet.</p> <p>B.II.b.2.b.3 - Change to batch release arrangements and quality control testing of the FP - Including batch control/testing for a biol/immunol product and one of the test methods is a biol/immunol/immunochemical</p>	20/09/2012	24/10/2012

A Reminder Change is Inevitable#3

Glybera (Gene Therapy)

II/0024	Change to active substance testing. B.I.b.2.d - Change in test procedure for AS or starting material/reagent/intermediate - Substantial change to or replacement of a biological/immunological/immunochemical test method or a method using a biological reagent for a biological AS	18/12/2013	n/a
		Changes, 003,004,005, 006, 008, 010,019 all to test methods	
II/0029	Change in finished product specification. B.II.d.1.e - Change in the specification parameters and/or limits of the finished product - Change outside the approved specifications limits range	23/01/2014	n/a
II/0025	C.I.4 - Change(s) in the SPC, Labelling or PL due to new quality, preclinical, clinical or pharmacovigilance data	20/03/2014	

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eals

(Reuters) - The Western world's first gene therapy drug is set to go on sale in [Germany](#) with a 1.1 million euro (\$1.4 million) price tag, a new record for a medicine to treat a rare disease.

Exclusive: First gene therapy drug sets million-euro price record

BY LUDWIG BURGER AND BEN HIRSCHLER

FRANKFURT/LONDON | Wed Nov 26, 2014 12:52pm EST

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An operator installs a chromatography column to purify the gene therapy drug Glybera at Dutch biotech company uniQure in Amsterdam December 13, 2012.

CREDIT: REUTERS/MICHAEL KOOREN

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