

ALLIANCE_{for}
Regenerative Medicine

Regenerative Medicine Clinical and Financial Overview

2014



About The Alliance for Regenerative Medicine

The Alliance for Regenerative Medicine (ARM) is a Washington, DC-based multi-stakeholder advocacy organization that promotes legislative, regulatory and reimbursement initiatives necessary to facilitate access to life-giving advances in regenerative medicine. ARM also works to increase public understanding of the field and its potential to transform human healthcare, providing business development and investor outreach services to support the growth of its member companies and research organizations. Prior to the formation of ARM in 2009, there was no advocacy organization operating in Washington, DC to specifically represent the interests of the companies, research institutions, investors and patient groups that comprise the entire regenerative medicine community. Today ARM has more than 150 members and is the leading global advocacy organization in this field. To learn more about ARM or to become a member, visit www.alliancerm.org.

Information in this report is from March 2013 through March 2014.

Table of Contents

Regenerative Medicine 2014

Geographic Breakdown	2
Industry Sector Breakdown	3

Clinical

Regenerative Medicine and Advanced Therapy Clinical Overview.	4
Primary Cell-Based Therapeutics	6
Stem and Progenitor Cell-Based Therapeutics.....	8
Cell-Based Immunotherapies.....	10
Gene Therapies.....	11
Clinical Breakdown by Therapeutic Category	12

Finance

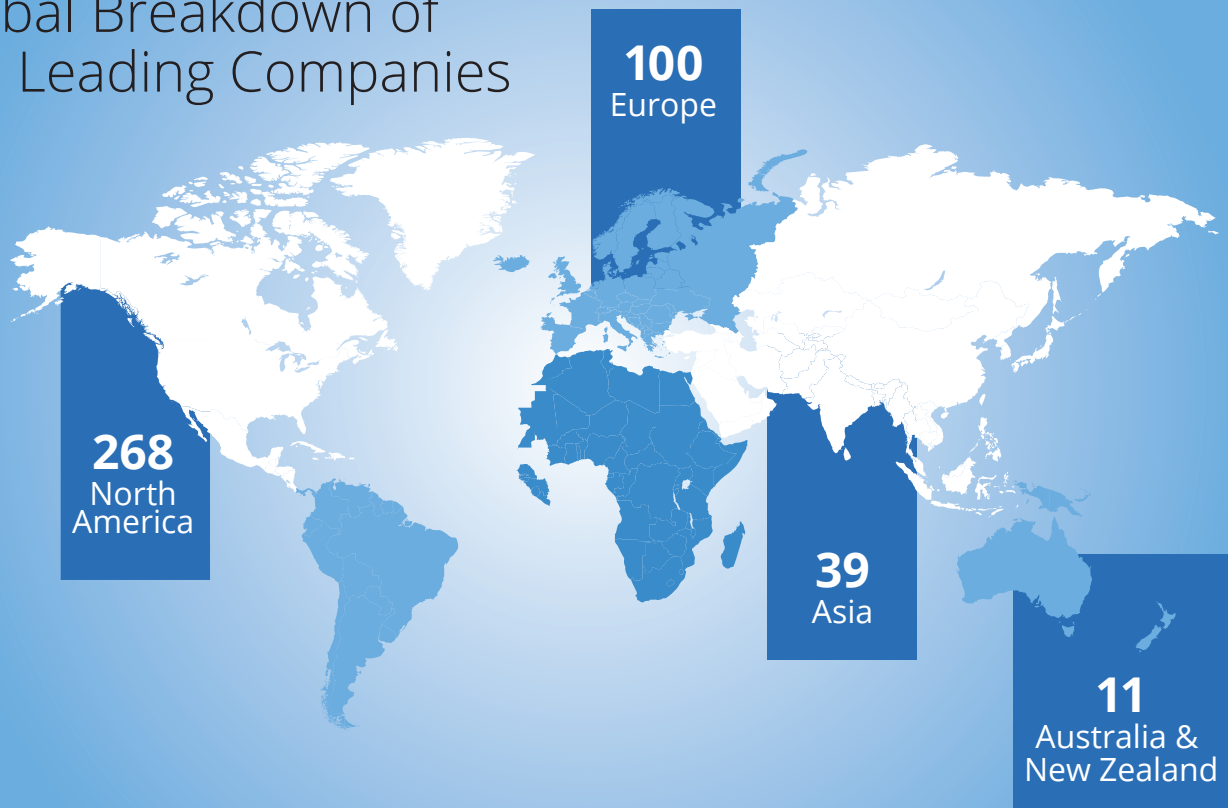
Regenerative Medicine Cell and Gene Therapy Financial Performance	20
Noteworthy Deals and Acquisitions	21
Noteworthy Financings	22

ARM Membership	24
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Regenerative Medicine Industry 2014

It's estimated that there are more than 700 companies worldwide with a regenerative medicine focus ranging from divisions of multinational corporations to smaller firms focused on niche products or platform technologies. The Alliance for Regenerative Medicine (ARM) is actively tracking 418 of these leading companies. This report covers the clinical activity and trends of 247 therapeutically-focused regenerative medicine and advanced therapy companies developing cell therapies, gene therapies and gene-modified cellular therapies.

Global Breakdown of 418 Leading Companies



ARM's Definition of Regenerative Medicine

Regenerative medicine research translates fundamental knowledge in biology, chemistry and physics into materials, devices, systems and a variety of therapeutic strategies, which augment, repair, replace or regenerate organs and tissue.

These therapies represents a new paradigm in human health with the potential to resolve unmet medical needs through technologies that are specifically developed to modify diseases and enable tissue regeneration or replacement. This includes cells, biologics, genes or drugs controlling pathways related to disease causation in addition to regulating tissue regeneration, as well as synthetic or natural scaffolds for tissue engineering.

Company Breakdown by Industry Sectors



56%
Therapeutics
& Devices



19%
Tools



13%
Banks



12%
Services

The majority of regenerative medicine companies are therapeutically focused and developing a variety of technologies including cell-based therapies, small molecule and biologic based therapies, gene therapies, tissue-engineered biomaterials and scaffolds and implantable devices. The second largest group of regenerative medicine companies are developing tools such as stem cells for drug discovery and toxicity testing, as well as clinical tools, bioprocessing tools and platforms that include equipment, consumables, reagents and storage systems to support commercialization and clinical applications. The field also incorporates a variety of service companies specializing in clinical trial management, manufacturing, characterization, engineering and quality control, among others. For the purpose of this report we will concentrate on two therapeutic pillars where the majority of clinical activity is occurring—cell and gene therapies.



Cell Therapies

Primary cell-based therapies represent the most mature sub-sector of regenerative medicine. These therapies are composed of terminally differentiated adult cells isolated directly from human tissue including fibroblasts, keratinocytes, chondrocytes, osteoblasts, myocytes, hepatocytes, leukocytes, lymphocytes and endothelial cells, to name a few.

Stem cell and progenitor cell therapies represent the largest therapeutic sub-sector of the regenerative medicine industry. These therapies are based on a variety of different stem cell types including hematopoietic stem cells, mesenchymal stem cells, neural stem cells, epithelial stem cells, embryonic stem cells and more recently, induced pluripotent stem cells.

First generation cell-based immunotherapy products are based on interleukins, cytokines, chemokines, etc., but an emerging class are cell-based immunotherapies that employ lymphocytes, macrophages, dendritic cells, T-cells, natural killer cells and cytotoxic T lymphocytes. Some are autologous, while others are allogeneic, and many are being genetically modified to induce the desired immune response.



Gene Therapies

Gene therapies address defective or mutated genes needing either correction or improved regulation through the insertion of properly functioning or genetically altered genes into a patient's cells. The largest segment of gene therapies targets cancer, however regenerative-focused gene therapies are being developed for HIV and other infectious diseases, ocular disease, cardiovascular disease as well as several monogenic diseases.

Regenerative Medicine and Advanced Therapy Overview



247

Total Therapeutic

COMPANIES



466

Total Unique Therapeutic

PRODUCTS



699

Total Preclinical & Clinical Stage

TRIALS

Primary Cell-Based Therapeutics



45 Companies



70 Unique Products



17 Marketed Products



94

Trials

Stem Cell and Progenitor Cell-Based Therapeutics



91 Companies



154 Unique Products



9 Marketed Products



289

Trials

Cell-Based Immunotherapies



42 Companies



137 Unique Products



1 Marketed Product



173

Trials

Gene Therapies



69 Companies



105 Unique Products



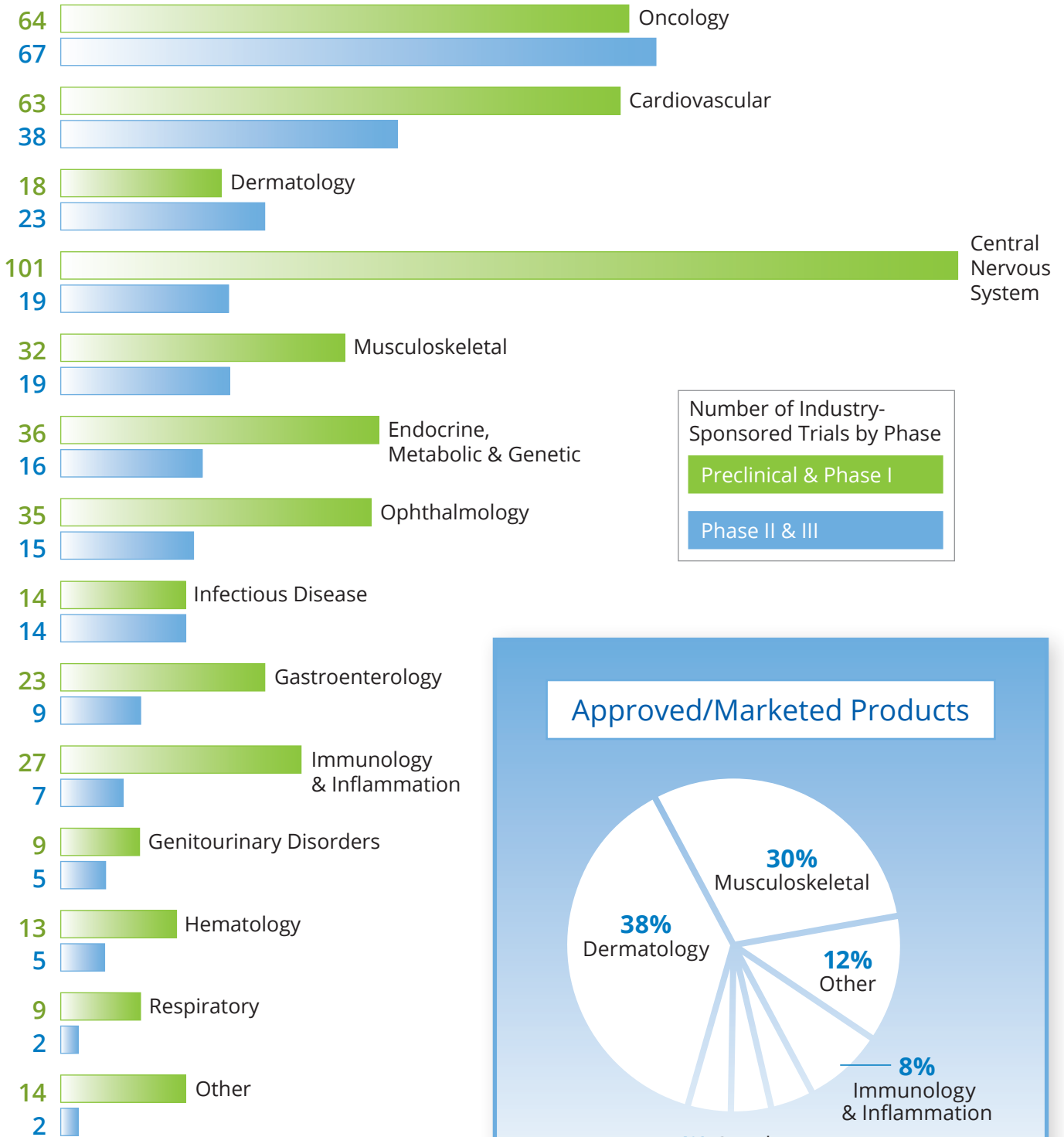
1 Approved Product
uniQure's Glybera



143

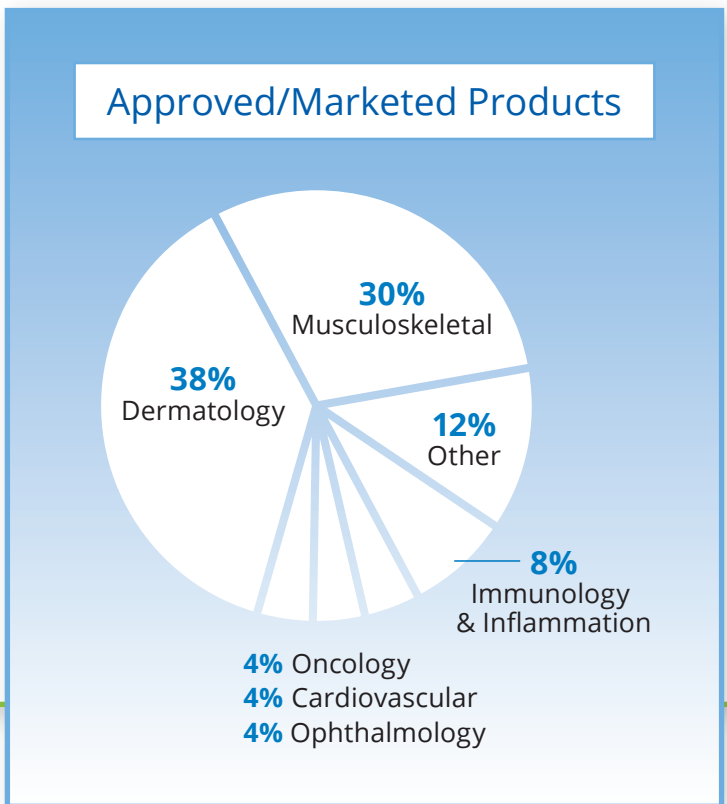
Trials

Regenerative Medicine and Advanced Therapy Clinical Breakdown by Therapeutic Areas

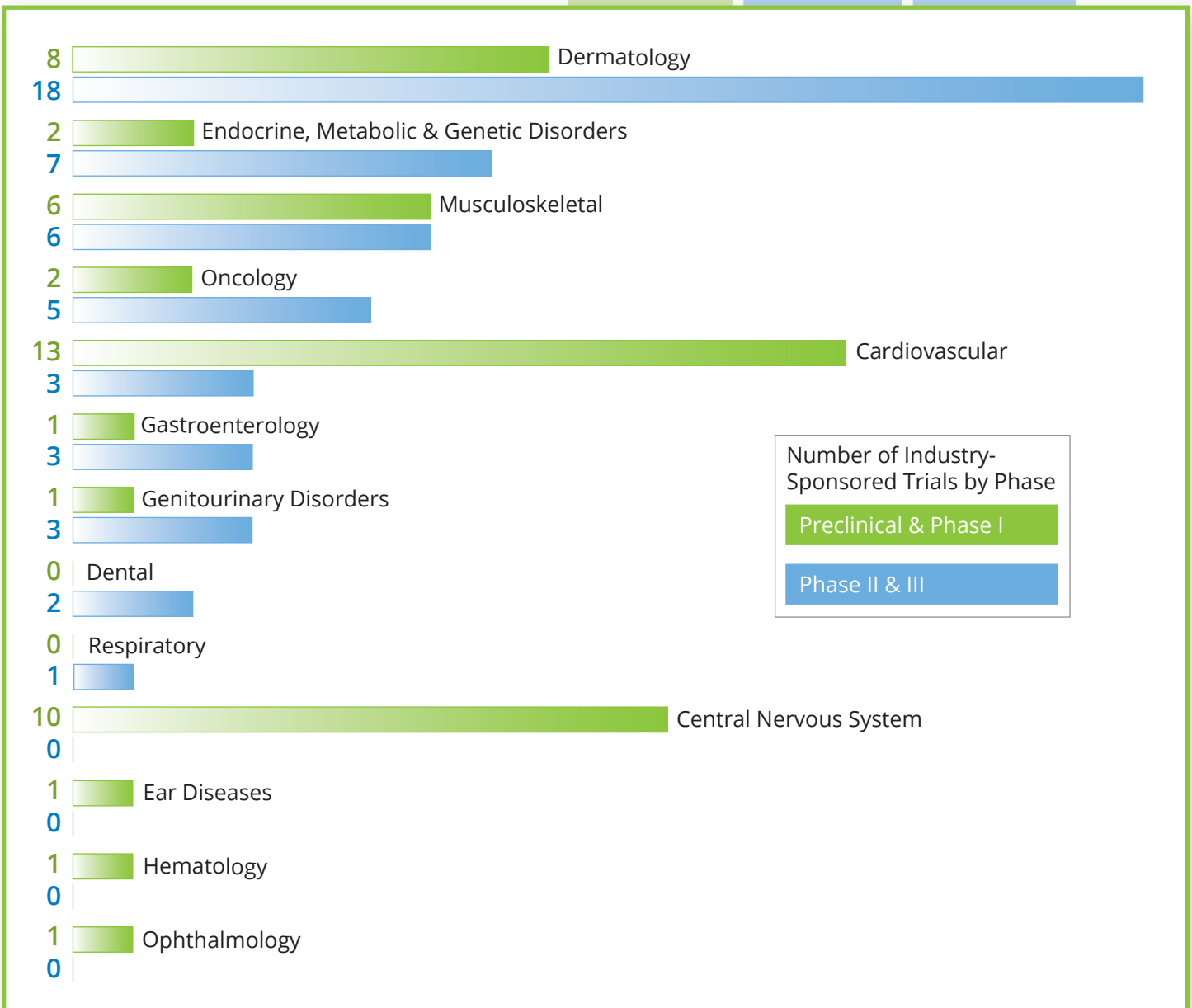
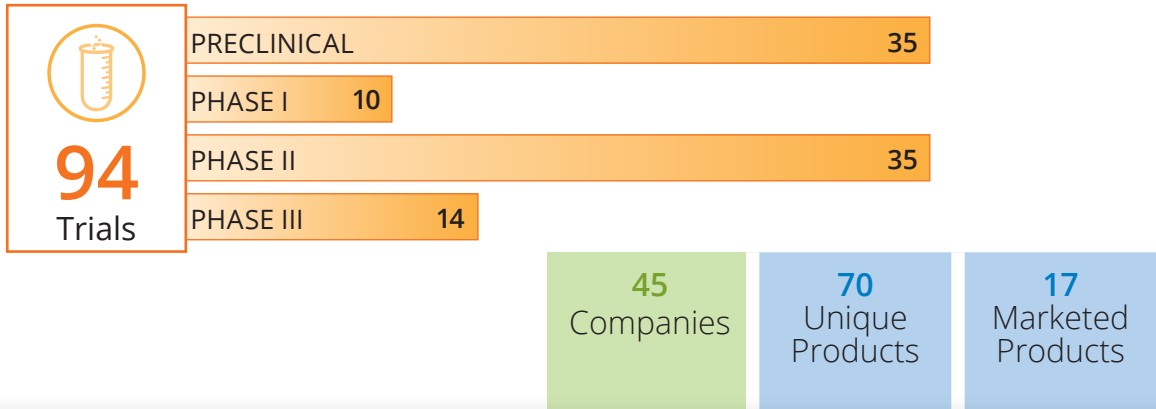


Number of Industry-Sponsored Trials by Phase

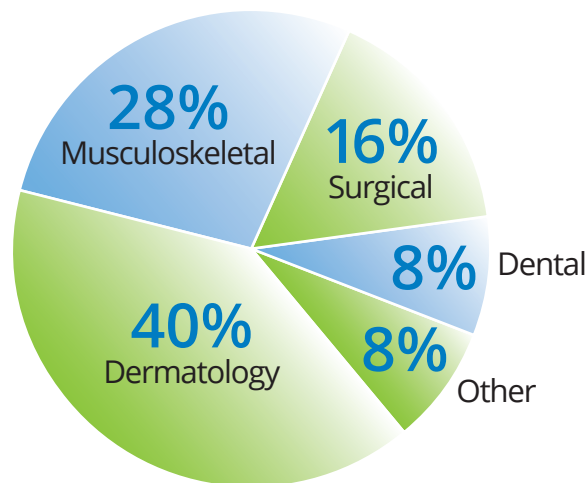
- Preclinical & Phase I
- Phase II & III



Primary Cell-Based Therapeutic Companies



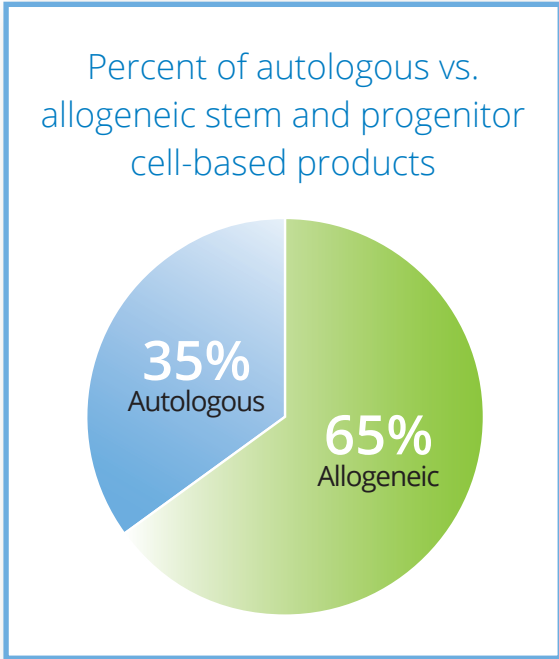
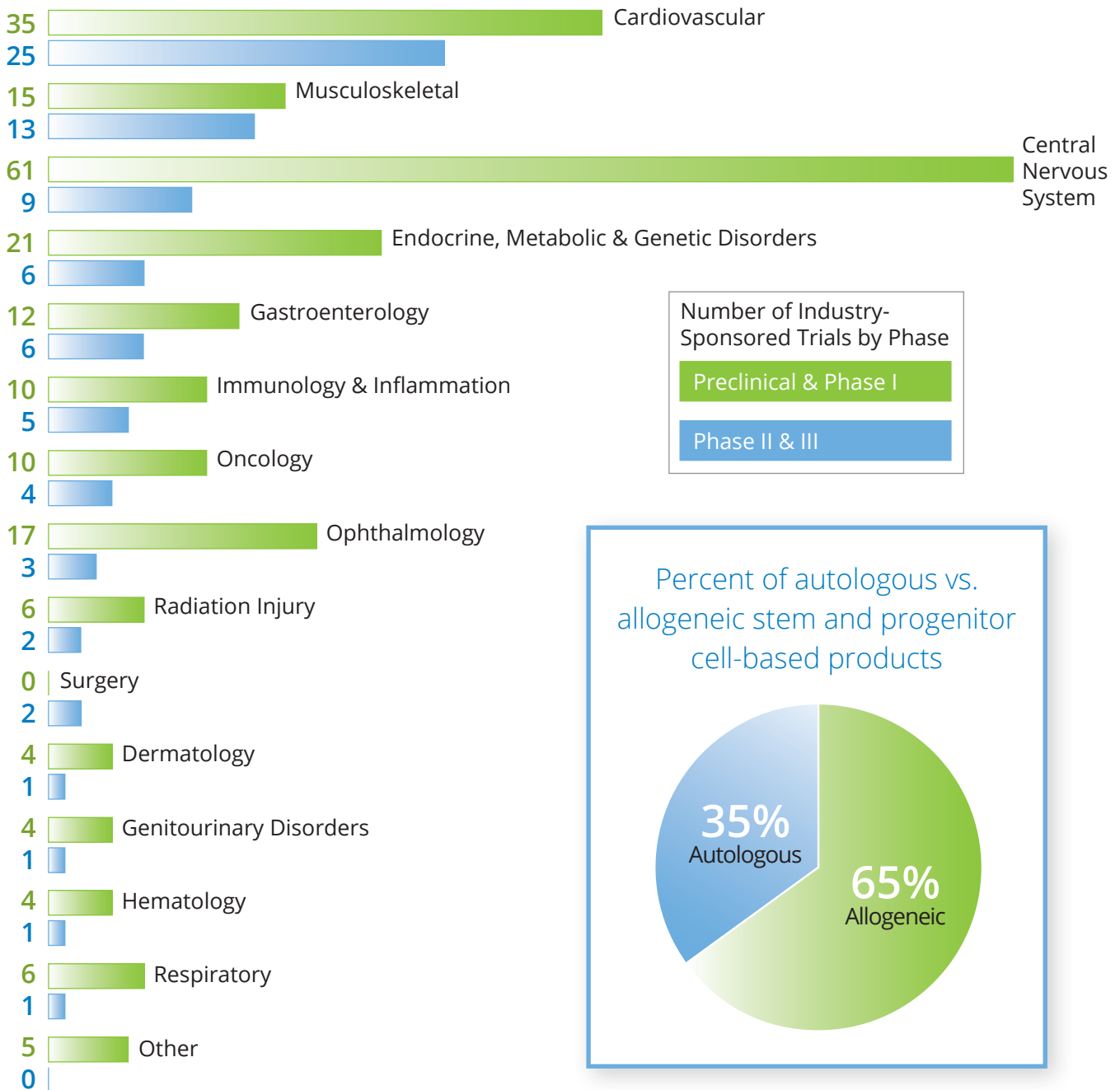
Therapeutic Breakdown of Commercially Available Primary Cell Therapies and Allograft Products



A Sampling of Commercial Primary Cell Therapy and Allograft Products

Company	Product	Indication
Avita Medical	ReCell	Dermatology
BioD	BioDFence	Surgical Protection
BioD	BioDFactor	Wound Healing
Educell d.o.o.	UroArt	Vesicoureteral Reflux
Fibrocell Science	azficel-T	Dermatology
Genzyme, a Sanofi Company	Carticel, Carticel Plus	Cartilage Defects
Genzyme, a Sanofi Company	Epicel	Burns
MacroCure	CureXcell	Wound Healing
MiMedx Group, Inc.	EpiFix	Wound Healing
Organogenesis Inc.	Dermagraft	Diabetic Foot Ulcer
Organogenesis Inc.	Gintuit	Mucogingival Conditions
Organogenesis Inc.	Apligraf	Diabetic Foot Ulcer
Orthofix	Trinity ELITE/Trinity Evolution	Musculoskeletal Defects
Osiris Therapeutics	Grafix	Wound Healing
TETEC Tissue Engineering Technologies	Novocart Inject, Novocart 3D, Novocart Disc	Cartilage Defects
TiGenix NV	ChondroCelect	Cartilage Defects

Stem and Progenitor Cell-Based Companies and Products



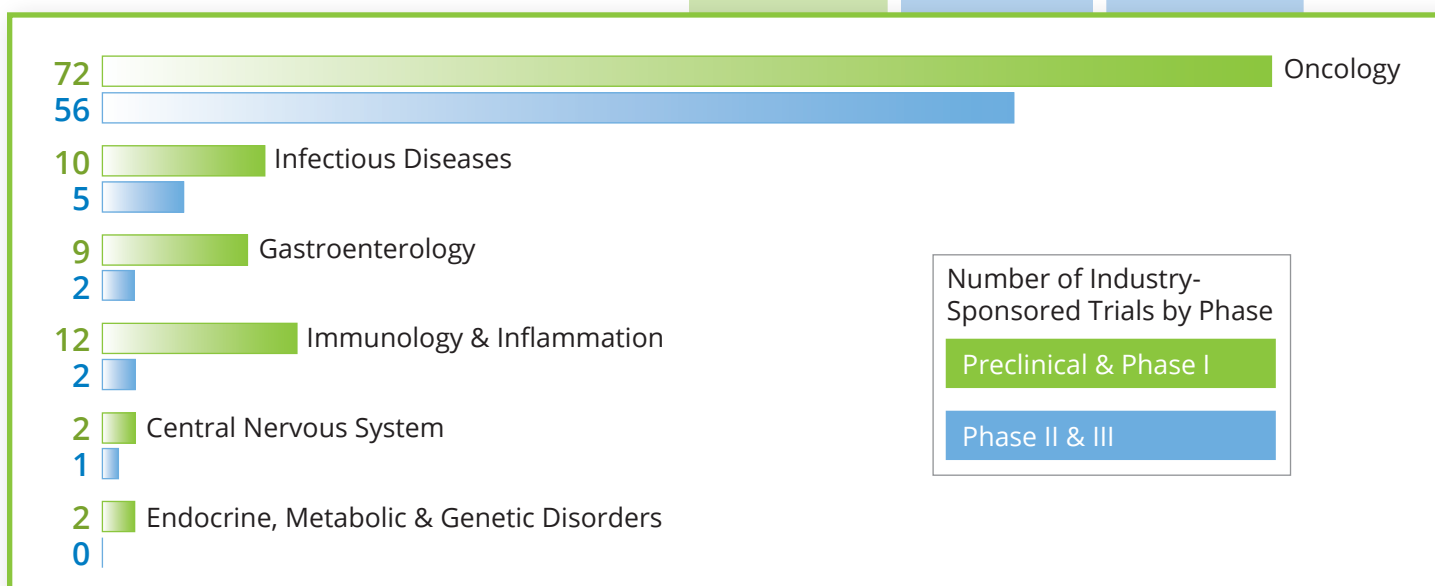
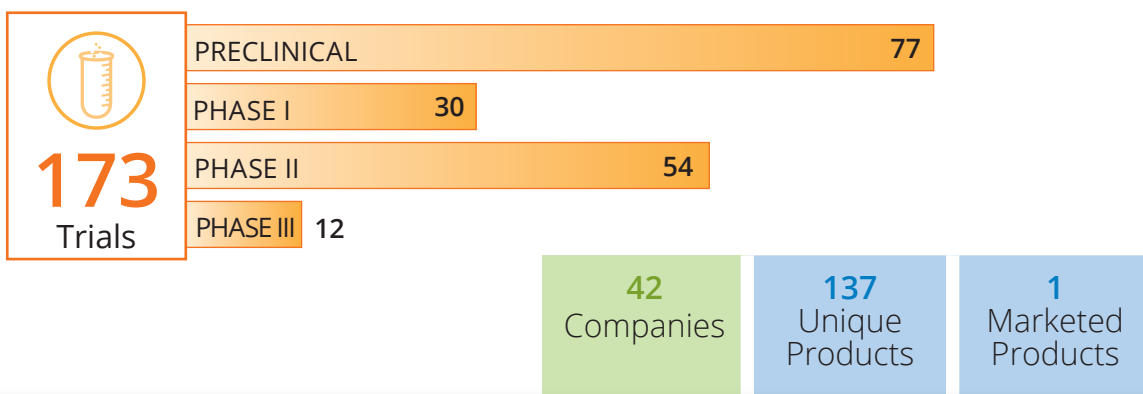
A Sampling of Trials with Expected 2014 Clinical Readouts

Company	Product	Indication	Milestone	Estimated Date
Athersys, Inc.	MultiStem	Ulcerative Colitis	Phase II trial result	2Q14
Athersys, Inc.	MultiStem	Ischemic Stroke	Preliminary Phase II trial result	2Q14
Cytomedix	ALD-401	Ischemic Stroke	Phase II trial result	2Q14
Cytori Therapeutics, Inc.	ADRCs	Ischemia	Phase II trial result	2Q14
Kiadis Pharma	ATIR	Hematological Malignancies	Phase II trial result	2Q14
Neuralstem Inc.	NSI-566	Amyotrophic Lateral Sclerosis	Phase II trial result	2Q14
NeoStem	AMR-001	Myocardial Infarction	Phase II trial result	3Q14
StemCells, Inc.	HuCNS-SC	Spinal Cord Injuries	Additional Phase I/II trial result	3Q14
ReNeuron Group plc	ReN009	Critical Limb Ischemia	Phase I trial result	4Q14
StemCells, Inc.	HuCNS-SC	Dry Age-Related Macular Degeneration	Preliminary Phase I/II trial result	4Q14
Tengion, Inc.	Neo-Kidney Augment	Chronic Kidney Failure	Preliminary Phase I trial result	4Q14
TiGenix NV	Cx601	Perianal Fistula	Final Phase III trial result	4Q14

A Sampling of IND Filings Expected in 2014

Company	Product	Indication	Expected Filing Date
NeoStem	VSELS	Periodontitis	2Q14
ViaCyte, Inc.	VC-01	Insulin-Dependent Diabetes Mellitus	3Q14
ReNeuron Group plc	ReN003	Retinitis Pigmentosa	3Q14
International Stem Cell Corporation	human parthenogenetic stem cells	Parkinson's Disease	3Q14
NeoStem	AMR-001	Congestive Heart Failure	4Q14
Q Therapeutics	Q-Cells	Amyotrophic Lateral Sclerosis	4Q14

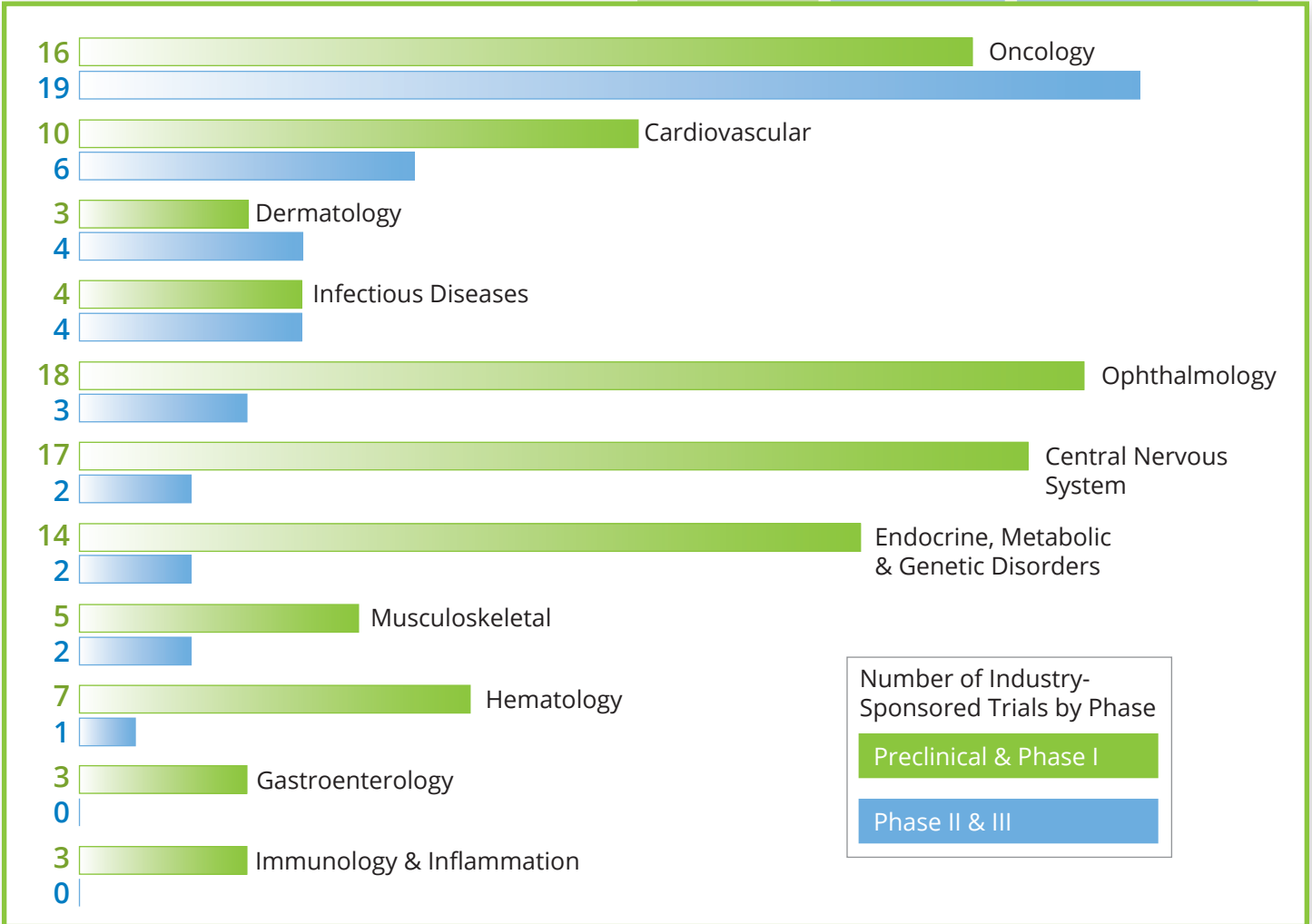
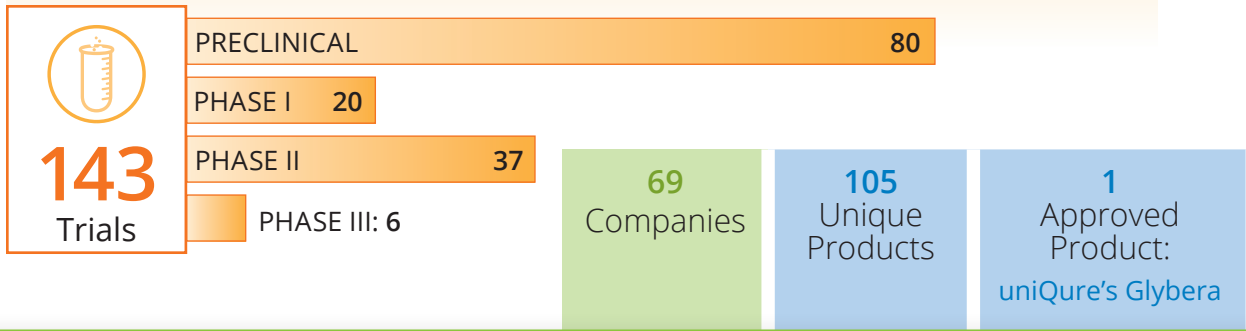
Cell-Based Immunotherapy Companies



A Sampling of Trials with Expected 2014 Clinical Readouts

Company	Product	Indication	Milestone	Estimated Date
Prima BioMed	CVac-CAN-003	Ovarian Cancer	Final Phase II trial analysis	2Q14
Northwest Biotherapeutics, Inc.	DCVax-Direct	Solid Tumors	Phase I/II trial result	2Q14
Immunocellular Therapeutics, Ltd.	ICT-107	Glioblastoma Multiforme	Additional Phase II trial result	3Q14
Argos Therapeutics, Inc.	AGS-004	Human Immunodeficiency Virus Infection	Phase IIb trial result	3Q14
Northwest Biotherapeutics, Inc.	DCVax-L	Glioblastoma Multiforme	Phase III trial result	4Q14
Adaptimmune Limited	NY-ESO-1/LAGE-1	Multiple Myeloma	Final Phase I/II trial analysis	4Q14

Gene Therapy Companies



A Sampling of Trials with Expected 2014 Clinical Readouts

Company	Product	Indication	Milestone	Estimated Date
uniQure NV	Glybera	Hyperlipoproteinemia	European Launch	3Q14
Taxus Cardium	Generx	Ischemic Heart Diseases	Phase III trial analysis	3Q14
Juventas Therapeutics	JVS-100	Cardiovascular Failure	Phase II trial result	4Q14
Juventas Therapeutics	JVS-100	Critical Limb Ischemia	Phase II trial result	4Q14

Regenerative Medicine and Advanced Therapy Clinical Trial Breakdown by Therapeutic Category

A sampling of cell and gene therapy companies in clinical stages of development

Musculoskeletal

Company	Product / Indication	Phase I	Phase II	Phase III
Bone Therapeutics SA	PREOB / Avascular Necrosis, Bone Fracture			
CellCoTec	INSTRUCT / Articular Cartilage Lesion of the Knee			
Cellular Biomedicine Group	Rejoin / Osteoarthritis			
Co.don AG	co.don chondrosphere / Articular Cartilage Lesion of the Femoral Condyle			
Cytori Therapeutics, Inc.	ADRCs / Hamstring Injury			
DePuy Mitek, Inc.	CAIS / Defect of Articular Cartilage			
Histogenics	NeoCart / Articular Cartilage Damage			
ISTO Technologies	DeNovoET / Articular Cartilage Damage			
ISTO Technologies	NuQu / Degenerative Disc Disease			
Mesoblast Ltd.	MPCs / Spinal Fusion			
Mesoblast Ltd.	MPCs / Intervertebral Disc Repair			
Pluristem Therapeutics Inc.	PLX-PAD / Muscle Injury			
RepliCel	RCT01 / Achilles Tendonitis			
Tissue Engineering Technologies AG	Novocart Disc Plus / Lumbar Degenerative Disc Disease			
TissueGene, Inc.	TissueGene-C / Degenerative Arthritis			
TissueGene, Inc.	TissueGene-C / Knee Osteoarthritis			

Musculoskeletal-related conditions in the U.S. account for 132 million visits to physicians' offices, 29 million visits to emergency rooms, 15 million hospital outpatient visits and cost over \$850 billion each year. Further, musculoskeletal injuries in the U.S. cause workers to miss more than 440 million days of work annually.

Source: Penn Center for Musculoskeletal Disorders, *Overview of the Penn Center for Musculoskeletal Disorders*, Perelman School of Medicine website, <http://www.med.upenn.edu/pcmd/overview.shtml>

Cardiovascular/Vascular Disease

Company	Product / Indication	Phase I	Phase II	Phase III
Amorcyte/NeoStem	AMR001 / Myocardial Infarction			
Athersys, Inc.	MultiStem / Myocardial Infarction			
Bioheart, Inc.	LipiCell / Congestive Heart Failure			
Bioheart, Inc.	MyoCell / Congestive Heart Failure			
Capricor Therapeutics, Inc.	CAP1002 / Myocardial Infarction			
Capricor Therapeutics, Inc.	CAP1001 / Myocardial Infarction			
Cardio3 BioSciences	C3BS-CQR-1 / Heart Failure			
Celgene Corporation	PDA002 / Peripheral Arterial Disease			
Cytomedix	ALD201 / Ischemic Heart Diseases			
Cytomedix	ALD301 / Critical Limb Ischemia			
Cytomedix	ALD301 / Intermittent Claudication			
Cytori Therapeutics, Inc.	ADRCs / Myocardial Infarction			
Cytori Therapeutics, Inc.	ADRCs / Ischemia			
Harvest Technologies Corp.	BMAC / Critical Limb Ischemia			
Juventas Therapeutics	JVS100 / Congestive Heart Failure			
Juventas Therapeutics	JVS100 / Critical Limb Ischemia			
Mesoblast Ltd.	Revascor / Congestive Heart Failure			
Mesoblast Ltd.	Revascor / Myocardial Infarction			
Mesoblast Ltd.	Autologous MPCs / Ischemia			
Pharmicell	Hearticellgram-AMI / Acute Myocardial Infarction			
Pluristem Therapeutics Inc.	PLX-PAD / Intermittent Claudication			
Pluristem Therapeutics Inc.	PLX-PAD / Critical Limb Ischemia			
Stemmedica Cell Technologies, Inc.	Allogeneic Mesenchymal Bone Marrow Cells / Myocardial Infarction			
TotipotentRX/Cesca Therapeutics	Autologous bone marrow derived stem cells / Critical Limb Ischemia			
TotipotentRX/Cesca Therapeutics	Autologous bone marrow derived stem cells / Myocardial Infarction			

Between 2012 and 2030, total stroke-related costs are projected to triple, from \$71.6 billion to \$184.1 billion.

Source: American Heart Association, *Heart Disease and Stroke Statistics—2014 Update*, <http://circ.ahajournals.org/content/129/3/e28.full#ref-726>

Oncology

Company	Product / Indication	Phase I	Phase II	Phase III
Adaptimmune Limited	Autologous Genetically modified T cells / Multiple Myeloma			
Adaptimmune Limited	NY-ESO-1/LAGE-1 / Metastatic Melanoma, Multiple Myeloma, Ovarian Cancer			
Aduro Biotech	GVAX Leukemia Vaccine / Acute Myeloid Leukemia, Chronic Myeloid Leukemia			
Aduro Biotech	GVAX Pancreatic Cancer Vaccine / Pancreatic Cancer			
Advantagene, Inc.	AdVtk Therapy / Colon Cancer, Malignant Pleural Effusion, Mesothelioma, Ovarian Cancer			
Advantagene, Inc.	AdVtk Therapy / Glioma			
Advantagene, Inc.	ProstAtak / Local Prostate Cancer			
Advantagene, Inc.	PancAtak / Locally Advanced Pancreatic Cancer			
Argos Therapeutics, Inc.	AGS003 / Metastatic Renal Cell Carcinoma			
Argos Therapeutics, Inc.	AGS005 / Chronic Lymphoid Leukemia			
Argos Therapeutics, Inc.	RNA-Loaded Dendritic Cell Vaccine / Melanoma			
Bellicum Pharmaceuticals, Inc.	BPX101 with AP1903 / Hormone Refractory Metastatic Prostate Cancer			
Bellicum Pharmaceuticals, Inc.	BPX501 / Hematological Malignancies			
California Stem Cell/NeoStem	Tumor Stem Cell Specific Dendritic Cell Therapy / Renal Cell Carcinoma			
Cellerant Therapeutics, Inc.	CLT008 / Cancer Chemotherapy Induced Neutropenia			
Coronado Biosciences	CNDO109 / Acute Myeloid Leukemia			
DCPrime B.V.	DCP001 / Acute Myeloid Leukemia			
Dendreon Corporation	Provenge / Local Prostate Cancer			
Dendreon Corporation	Provenge / Prostate Cancer			
Erytech Pharma	GRASPA / Acute Myeloid Leukemia			
Fate Therapeutics	ProHema / Hematological Malignancies			
Gamida Cell	NiCord / Hematological Malignancies			
Gamida Cell	StemEx / Hematological Malignancies			
Glycostem	Natural Killer Cells / Acute Myeloid Leukemia			
Immunocellular Therapeutics, Ltd.	ICT121 / Glioblastoma Multiforme			
Kite Pharma	Engineered Adoptive Cell Therapy / Non-Hodgkin's Lymphoma			
Lentigen Corporation	LG723 / Melanoma			
Lentigen Corporation	LG740 / Hematological Malignancies			

Oncology continued

Company	Product / Indication	Phase I	Phase II	Phase III
Lion Biotechnologies	Contego / Breast Cancer, Colorectal Cancer, Metastatic Melanoma, Ovarian Cancer			
MolMed S.p.A.	HSV-TK / Hematological Malignancies			
NewLink Genetics, Inc.	HyperAcute Melanoma Immunotherapy / Melanoma			
NewLink Genetics, Inc.	HyperAcute Prostate Cancer Vaccine / Hormone Refractory Prostate Cancer			
NewLink Genetics, Inc.	HyperAcute Prostate Cancer Vaccine / Metastatic Renal Cell Carcinoma			
NewLink Genetics, Inc.	HyperAcute Pancreatic Cancer Vaccine / Pancreatic Cancer			
NewLink Genetics, Inc.	HyperAcute Lung Immunotherapy / Non-small Cell Lung Cancer			
Northwest Biotherapeutics, Inc.	DCVax-L / Brain Cancer			
Northwest Biotherapeutics, Inc.	DCVax Direct / Solid Tumors			
Northwest Biotherapeutics, Inc.	DCVax Direct / Metastatic Colon Cancer			
Northwest Biotherapeutics, Inc.	DCVax L Ovarian / Metastatic Ovarian Cancer			
Northwest Biotherapeutics, Inc.	DCVax Prostate / Hormone Refractory Prostate Cancer			
Oxford BioMedica	MetXia with Cyclophosphamide / Breast Cancer, Melanoma, Pancreatic Cancer			
Oxford BioMedica	TroVax / Colorectal Cancer, Hormone Refractory Prostate Cancer, Mesothelioma			
Oxford BioMedica	TroVax / Metastatic Renal Cell Carcinoma			
Sangamo BioSciences	SB313 / Glioblastoma Multiforme			
SOTIO	Dcvac/Pca / Hormone Refractory Metastatic Prostate Cancer, Local Prostate Cancer, Metastatic Prostate Cancer			
Tengion, Inc.	Neo-Urinary Conduit / Bladder Cancer			
Vical, Inc.	Leuvectin / Metastatic Melanoma, Sarcomas			

Infectious Diseases

Company	Product / Indication	Phase I	Phase II	Phase III
Argos Therapeutics, Inc.	AGS004			
Calimmune	Cal-1			
Cell Medica	Cytovir ADV			
Cell Medica	Cytovir CMV			
Sangamo BioSciences	SB-728			

Endocrine, Metabolic and Genetic Disorders

Company	Product / Indication	Phase I	Phase II	Phase III
Cytomedix	ALD601 / Lysosomal Storage Disorders			
Living Cell Technologies Limited	DIABECELL / Type 1 Diabetes			
Mesoblast Ltd.	MPCs / Diabetic Nephropathy			
Mesoblast Ltd.	MPCs / Non-Insulin-Dependent Diabetes Mellitus			
NeoStem	Treg Program / Insulin-Dependent Diabetes Mellitus			
Oxford BioMedica	UshStat / Usher syndrome type 1B			
Promethera	Hepastem / Metabolic Disorders			
Promethera	Hepastem / Urea Cycle Disorder			
t2cure	t2c002/t2c003 / Diabetic Neuropathy			

Diabetes affects 25.8 million people or 8.3% of the U.S. population. Total direct and indirect medical cost for diabetes care in the U.S. as of 2007 equaled \$174 billion—\$116 billion of that was direct medical costs.

Source: Centers for Disease Control and Prevention, 2011 National Diabetes Fact Sheet, Diabetes Public Health Resource, <http://www.cdc.gov/diabetes/pubs/factsheet11/fastfacts.htm>

Genitourinary Disorders

Company	Product / Indication	Phase I	Phase II	Phase III
AlloCure	AC607 / Acute Kidney Failure			
Cook Myosite	AMDC / Stress Urinary Incontinence			
Cytonet Group	HHLivC / Urea Cycle Disorders			
Innovacell Biotechnologie AG	ICES13 / Stress Urinary Incontinence			
Tengion, Inc.	Neo-Kidney Augment / Chronic Kidney Failure			
uniQure NV/Digna Biotech	AAV5-PB6D / Acute Intermittent Porphyria			

Gastroenterology

Company	Product / Indication	Phase I	Phase II	Phase III
Athersys, Inc.	MultiStem / Ulcerative Colitis			
Celgene Corporation	PDA001 / Crohn's Disease			
Innovacell Biotechnologie AG	ICEF15 / Fecal Incontinence			
Mesoblast Ltd.	Prochymal / Crohn's Disease			
TiGenix NV	Cx601 / Perianal Fistula			
TxCell SA	OvaSave / Crohn's Disease			

In 2012, Americans spent and estimated \$200 billion to care for those with Alzheimer's, including \$140 billion to Medicare and Medicaid.

Unless something is done, the costs of Alzheimer's in 2050 are estimated to total \$1.1 trillion (in today's dollars). Costs to Medicare and Medicaid will increase nearly 500%.

Source: Alzheimer's Association, *March 2012 Fact Sheet*,
https://www.alz.org/documents_custom/2012_facts_figures_fact_sheet.pdf

Central Nervous System

Company	Product / Indication	Phase I	Phase II	Phase III
Athersys, Inc.	MultiStem / Ischemic Stroke			
bluebird bio	Lenti-D / Adrenoleukodystrophy			
BrainStorm Cell Therapeutics	SC Therapy / Amyotrophic Lateral Sclerosis			
Cytomedix	ALD401 / Ischemic Stroke			
MEDIPOST	Neurostem / Alzheimer's Disease			
Neuralstem Inc.	NSI567 / Spinal Cord Injuries			
Neuralstem Inc.	NSI566 / Amyotrophic Lateral Sclerosis			
Neuralstem Inc.	NSI566 / Ischemic Stroke			
NsGene A/S	NsG0202 / Alzheimer's Disease			
Oxford BioMedica	Prosavin / Idiopathic Parkinson's Disease			
REGENX Biosciences	CLN2 Gene Therapy / Batten Disease			
ReNeuron Group plc	ReN001 / Cerebral Ischemia			
SanBio, Inc.	SB623 / Ischemic Stroke			
Sangamo BioSciences	CERE-110 / Alzheimer's Disease			
StemCells, Inc.	HuCNS-SC / Batten Disease			
StemCells, Inc.	HuCNS-SC / Pelizaeus-Merzbacher Disease			
StemCells, Inc.	HuCNS-SC / Spinal Cord Injuries			
uniQure NV	AAV2-GDNF / Parkinson's Disease			

Ophthalmology

Company	Product / Indication	Phase I	Phase II	Phase III
Advanced Cell Technology	Retinal Pigmented Epithelial therapy / Stargardt's Disease			
AGTC	AAV2-sFLT01 / Wet Age-Related Macular Degeneration			
AGTC	AAVRPE65 / Leber's Congenital Amaurosis			
Avalanche Biotech	AVA101 / Wet Age-Related Macular Degeneration			
Neurotech	NT-501 / Retinitis Pigmentosa			
NightstaRx Limited	AAV.REP1 / Choroideremia			
Oxford BioMedica	RetinoStat / Wet Age-Related Macular Degeneration			
Oxford BioMedica	StarGen / Stargardt's Disease			
StemCells, Inc.	HuCNS-SC / Dry Age-Related Macular Degeneration			

The estimated annual total financial burden to the U.S. economy of four major adult vision problems (AMD, cataract, diabetic retinopathy and glaucoma), refractive errors, visual impairment and blindness is \$35.4 billion.

Source: *The Economic Impact of Vision Problems: The Toll of Major Adult Eye Disorders, Visual Impairment and Blindness on the U.S. Economy*, http://www.preventblindness.net/site/DocServer/Impact_of_Vision_Problems.pdf?docID=1321
(published by Prevent Blindness America, 2007)

Hematology

Company	Product / Indication	Phase I	Phase II	Phase III
bluebird bio	LentiGlobin / Beta Thalassemia			
bluebird bio	LentiGlobin / Sickle Cell Anemia			
Cell Medica	Cytorex EBV / Lymphoma			
Gamida Cell	NiCord / Sickle Cell Anemia			
REGENX Biosciences	Factor IX Gene therapy / Hemophilia B			
uniQure NV	AMT060 / Hemophilia B			
uniQure NV/Digna	AMT021 / Acute Intermittent Porphyria			

Dermatology

Company	Product / Indication	Phase I	Phase II	Phase III
Avita Medical	ReCell / Burn Scar			
Avita Medical	ReCell / Hypertrophic Scar			
Celgene Corporation	PDA002 / Diabetic Foot Ulcer			
Fibrocell Science	azficel-T / Burn Scar			
Histogen Inc.	Regenica / Androgenetic Alopecia			
Intercytex	ProtoDerm / Skin Ulcers			
Intercytex	Vavelta / Burns, Acne Scars, Contracture Scar, Epidermolysis Bullosa, Wrinkles			
Juventas Therapeutics	JVS100 / Surgical Wound			
MacroCure	CureXcell / Diabetic Foot Ulcer			
NeoStem	VSEs / Wound			
Stratatech Corporation	StrataGraft / Burns			
Taxus Cardium	Genedexa / Diabetic Foot Ulcer			

Immunology and Inflammation

Company	Product / Indication	Phase I	Phase II	Phase III
Acorda Therapeutics	Ampydin / Guillain-Barre Syndrome			
Argos Therapeutics, Inc.	AGS009 / Systemic Lupus Erythematosus			
Athersys, Inc.	MultiStem / Bone Marrow Transplantation			
Athersys, Inc.	MultiStem / Liver Transplantation			
Cellerant Therapeutics, Inc.	CLT008 / Cord Blood Transplants			
Kiadis Pharma	Reviroc / Bone Marrow Transplantation			
Kiadis Pharma	Rhitol / Graft-Versus-Host Disease			
MEDIPOST	Promostem / Graft-Versus-Host Disease			
Mesoblast Ltd.	Revascor / Bone Marrow Transplantation			
Opexa Therapeutics, Inc.	Tcelna / Secondary Progressive Multiple Sclerosis			
Pluristem Therapeutics Inc.	PLX-BMT / Bone Marrow Transplantation			
TiGenix NV	Cx611 / Rheumatoid Arthritis			

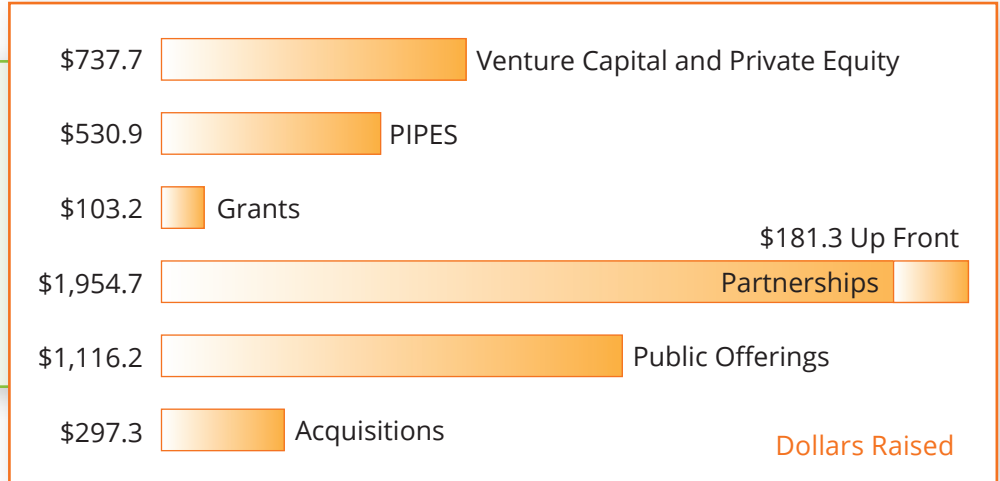
Regenerative Medicine Cell and Gene Therapy Financial Performance

Financings from March 2013 to March 2014.



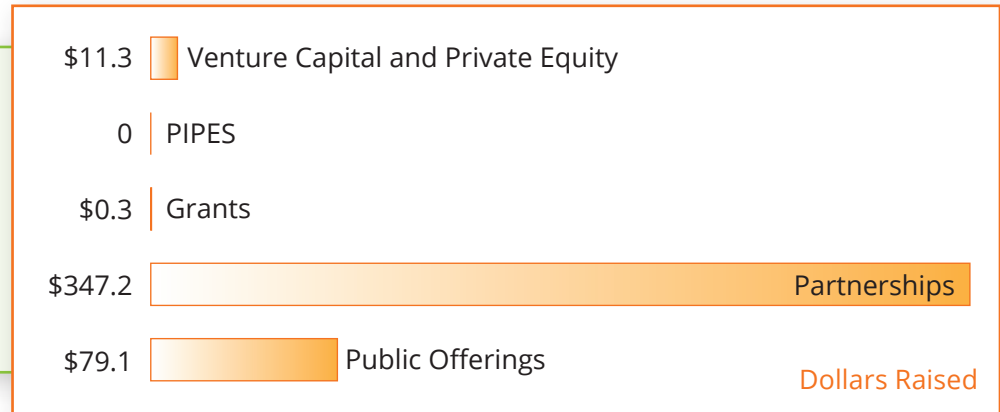
Combined
Regenerative
Medicine Field

\$4.74
Billion



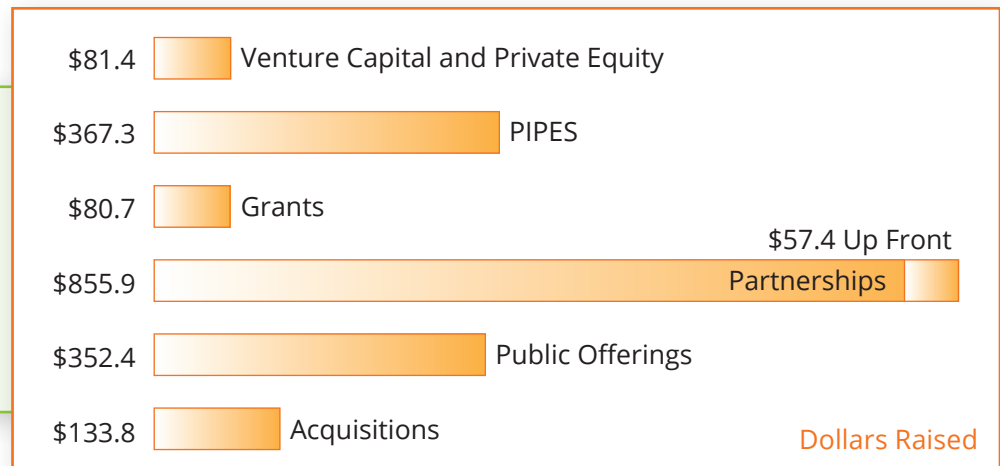
Primary Cell
Therapy

\$437.9
Million



Stem Cell and
Progenitor Cell
Therapy

\$1.872
Billion



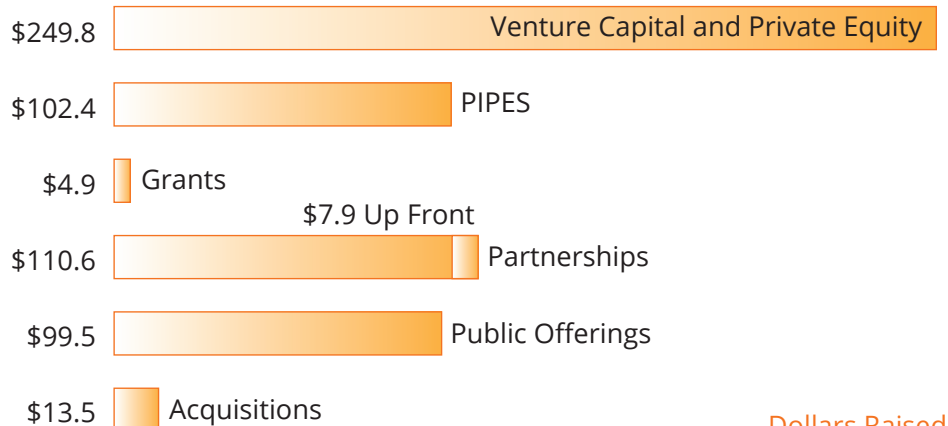
Noteworthy Deals and Acquisitions

Deal Type	Company(s)	Total Deal Value	Upfront Payment	Date
Collaboration	Tengion / Celgene	\$15M	\$15M	7/1/13
Merger	Capricor / Nile Therapeutics	NA	-	7/8/13
Commercialization Agreement	uniQure NV / Chiesi Farmaceutici	\$39.8M	\$39.8M	7/9/13
Collaboration	Stratatech / BARDA Contract	\$47.2M	-	7/31/13
Acquisition	Mesoblast / Osiris Stem Cell Therapeutic Business	\$100M	\$50M	10/11/13
Licensing Deal	Cytori Therapeutics, Inc. / Lorem Vascular	\$500M	\$24M	11/4/13
Licensing Deal	Pluristem / CHA Biotech	\$10.4M	\$10.4M	12/17/13
Acquisition	Intrexon / Medistem	\$26M	-	12/20/13
Collaboration/Licensing Deal	Capricor / J&J	\$325M	\$12.5M	1/6/14
Collaboration	Sangamo / Biogen Idec	\$320M	\$20M	1/9/14
Acquisition of Dermagraft	Organogenesis Inc. / Shire	\$300M	0	1/17/14
Acquisition	Sillajen / Jennerex	\$150M	-	3/17/14



Cell-Based
Immunotherapy

\$580.7
Million

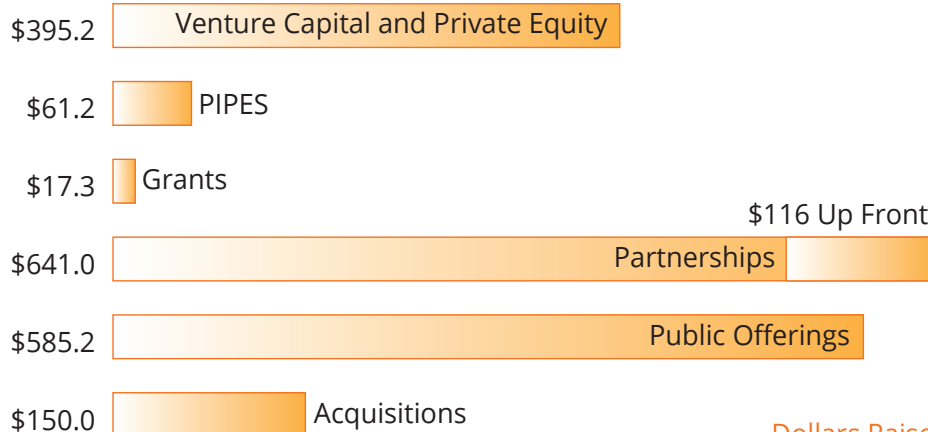


Dollars Raised



Gene
Therapy

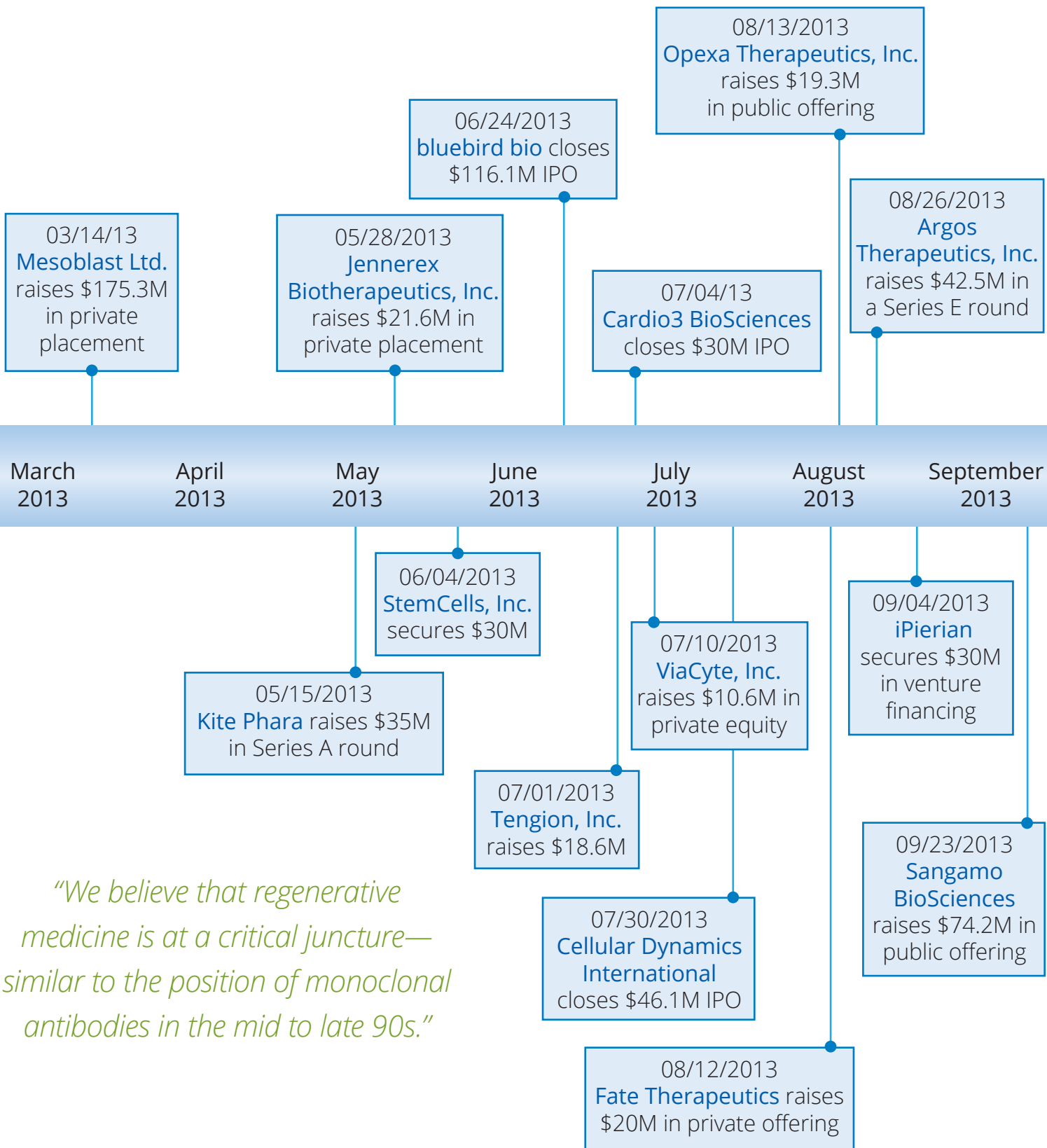
\$1.85
Billion



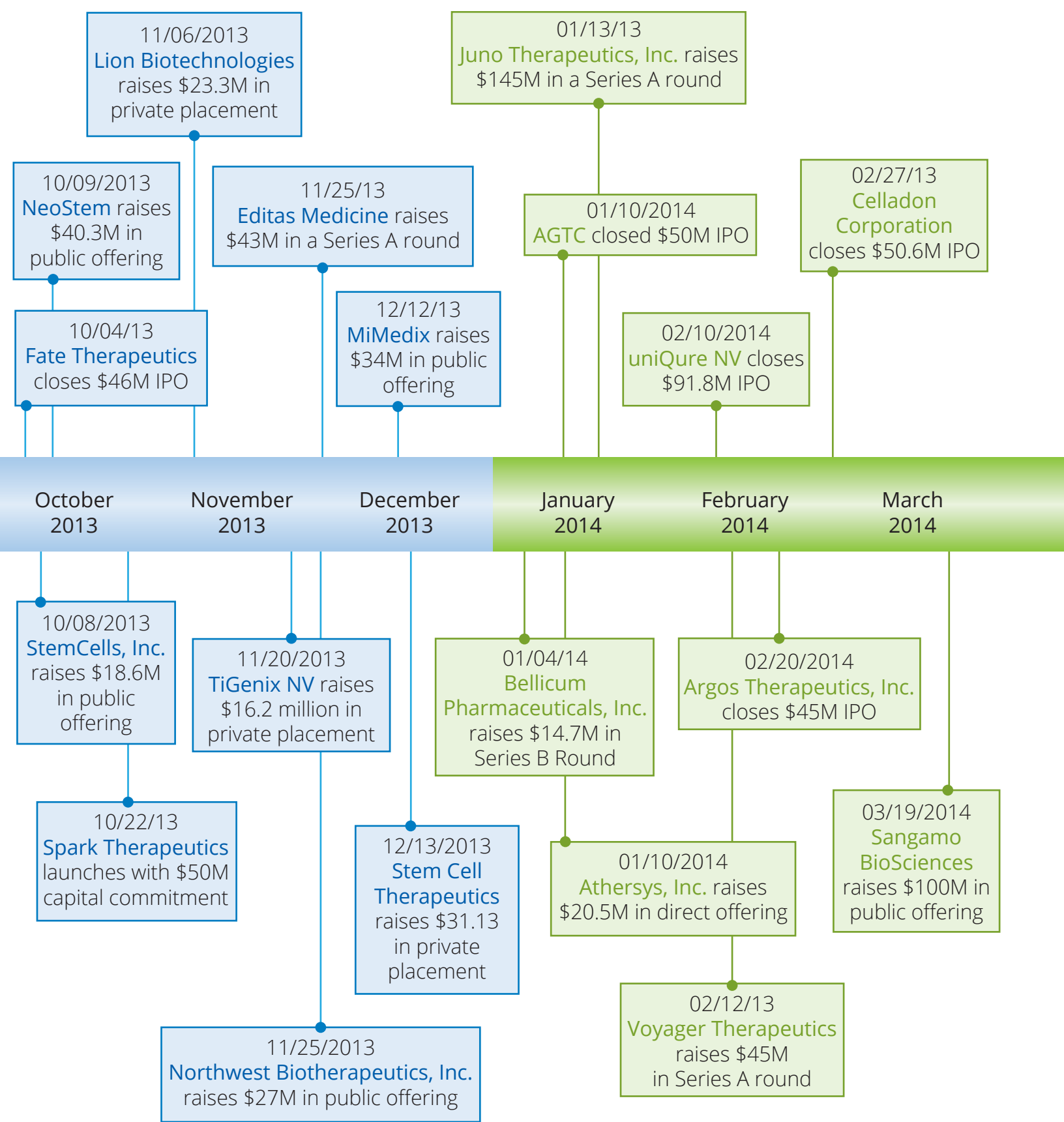
Dollars Raised

A Sampling of Noteworthy Financings

March 2013 through March 2014



"We believe that regenerative medicine is at a critical juncture—similar to the position of monoclonal antibodies in the mid to late 90s."



ALLIANCE^{for} *Regenerative Medicine*

Membership

Companies

Aastrom Biosciences, Inc.

Abeona Therapeutics

Advanced Cell Technology

AGTC

Akron Biotechnology

AlloCure

AlloSource

Athersys, Inc.

Avita Medical

AxoGen, Inc.

Baxter

Bell BioSystems

BioLife Solutions, Inc.

Biomatrix

Biospherix, Ltd.

BioTime, Inc.

Blood Centers of America Inc.

bluebird bio

BrainStorm Cell Therapeutics

Calimmune

Capricor Therapeutics, Inc.

Celgene Corporation

Cell Cure Neurosciences Ltd.

Cell Line Genetics, Inc.

Cell Therapy Group

CellGenix GmbH

Cellular Dynamics International

Cellular Technology Limited

Celsense Inc.

Cesca Therapeutics

Circle Biologics, Inc.

Cord Blood Registry

Clinical Trial & Consulting

Cynata Therapeutics Inc.

Cytomedix

Cytori Therapeutics, Inc.

DiscGenics, Inc.

Dohmen

EMD Millipore Corporation

Fate Therapeutics

Fibrocell Science

Fisher BioServices

GE Healthcare

GenVec

Global BioTherapeutics

Harvard Apparatus
Regenerative Technology

HemoGenix

Histogen Inc.

Histogenics

Humacyte, Inc.

Invetech

InvivoSciences, Inc.

iPierian Inc.

ISTO Technologies

Johnson & Johnson

Juventas Therapeutics

Lonza Group Ltd.

MaxCyte, Inc.

Medpace

Mesoblast Ltd.

MiMedx Group, Inc.

Minerva Biotechnologies
Corporation

Nanofiber Solutions

NeoStem

Northwest Biotherapeutics, Inc.

Organogenesis Inc.

Organovo Holdings, Inc.

OrthoCyte Corp.

Osiris Therapeutics

Oxford BioMedica

Pfizer Inc.

Pluristem Therapeutics Inc.

Progenitor Cell Therapy

Q Therapeutics

Regeneus Ltd.

Reglera

RepliCel

RhinoCyte Inc.

Rossi Group Consulting

RxGen

Sangamo BioSciences

Sanofi-Genzyme

Sartorius AG

Shire

SironRX Therapeutics

Smith & Nephew

StemBioSys

StemCells, Inc.

Tengion, Inc.

TERUMO BCT, Inc.

Thermo Fisher Scientific

Tissue Banks International

TissueGene, Inc.
Tissue Genesis, Inc.
TrakCel Ltd.
Vet-Stem, Inc.
ViaCyte, Inc.

Investors

Asset Management Ventures
Kentucky Seed Capital Fund
Novitas Capital
Toucan Capital
Triathlon Medical Ventures

Patient Advocates/ Foundations/Associations

Association of Clinical
Research Organizations
Alpha-1 Foundation
ALS Association
American Association for
Dental Research
BioBridge Global
California Institute for
Regenerative Medicine
Californians 4 Cures
Cell Society
Centre for Commercialization
of Regenerative Medicine
Friends of Cancer Research
Genetics Policy Institute
Human Organ Project, Inc.
International Society for
Stem Cell Research
JDRF
Missouri Cures

National Disease
Research Interchange
National Multiple Sclerosis Society
National Stem Cell Foundation
Nebraska Coalition for
Lifesaving Cures
New York Stem Cell Foundation
Parkinson's Action Network
Prevent Cancer Foundation
Stop ALD Foundation
Student Society for
Stem Cell Research
Texas Cures Education Foundation
Unite 2 Fight Paralysis

Research Institutions

Cleveland Clinic
Cornell University
Johns Hopkins Translational Tissue
Engineering Center
Neural Stem Cell Institute
Northwestern University
Comprehensive Transplant Center
Pittsburgh Tissue Engineering
Initiative
Sanford-Burnham Medical
Research Institute
Texas Heart Institute
UC San Diego Stem Cell Program
University of Maryland Center
for Stem Cell Biology and
Regenerative Medicine
University of Minnesota
Stem Cell Institute
University of Utah Cell Therapy and
Regenerative Medicine Program

Affiliates

AlphaMed Press

European Affiliates

Andalusian Initiative for
Advanced Therapies
Aposcience AG
ATMI
Cardio3 BioSciences
Cell2B
CellData Services
Cell Therapy Catapult
Chemelot Campus B.V.
DCPrime B.V.
Foundation for Biomedical
Research and Innovation
Fraunhofer Institute for
Cell Therapy and Immunology
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Kiadis Pharma
K.U. Leuven
Med Cell Europe AG
Newcastle University
Novadip Biosciences SA
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Promethera
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Center for Stem Cells and
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Voisin Consulting

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