Select Anticipated Clinical Data and Events

Last Updated: December 2018

Alliance for Regenerative Medicine

Company	Product	Therapeutic Modality	Indication	Clinical Stage	Expected Reporting Date
AveXis / Novartis	AVXS-101	Gene therapy	SMA Type 1	MAA filing, BLA submission, J-NDA filing (Japan)	Responses from EMA, FDA, MHLW (Japan) expected mid-2019
bluebird bio	Lentiglobin	Gene therapy	Transfusion dependent beta-thalassemia	MAA filing	Submitted MAA in 2H 2018; response expected 2019
Kiadis	ATIR101	Allodepleted T-Cell Immunotherapy	AML or ALL	Conditional EU approval	On track to receive CHMP opinion Q1 2019; EU launch anticipated 2H 2019
Mesoblast	Remestemcel-L	Allogeneic Mesenchymal Stem Cell Therapy	Acute Graft Versus Host Disease	BLA filing	preparation for BLA filing has commenced
Novartis	Zolgensma (formerly AVXS-101)	Gene therapy	SMA Type 1	BLA / MAA filing	Regulatory action from FDA anticipated May 2019; MAA decision in Japan expected mid-2019; MAA decision in EU expected mid-2019
Orchard Therapeutics	OTL-101	Gene therapy	ADA-SCID	MAA filing, BLA submission	Anticipates filing BLA, MAA in 2020
Orchard Therapeutics	OTL-200	Gene therapy	metachromatic leukodystrophy	MAA filing, BLA submission	Anticipates filing BLA, MAA in 2020
PTC Therapeutics	GT-AADC	Gene therapy	AADC Deficiency	BLA submission	Expects to submit BLA in 2019
Abeona	EB-101	Gene therapy	Recessive Dystrophic Epidermolysis Bullosa	Ph III	Initiate pivotal clinical trial in the middle of 2019
Atara Biotherapeutics	Tab-cel	Cell therapy	Epstein-Barr virus associated post-transplant lymphoproliferative disorder (EBV+ PTLD)	Ph III	Plan to submit conditional marketing authorization application in 2H 2019
Athersys	MultiStem	Cell therapy	Ischemic Stroke	Ph III (under SPA)	Ongoing

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BioCardia	CardiAMP cell therapy system	Autologous bone marrow derived cell therapy	Heart failure following heart attack	Ph III	Enrollment completed Q3 2019
BioMarin	Valoctocogene roxaparvovec	Gene therapy	Hemophilia A	Ph III	Increase in enrollment to 130 participants anticipated by 1Q 2019
bluebird bio	Lenti-D	Gene therapy	Cerebral Adrenoleukodystrophy	Ph III	To be initiated in 2019
bluebird bio	LentiGlobin	Gene therapy	Sickle cell disease	Ph III	To be initiated in 2019
Brainstorm	NurOwn	Mesenchymal Stem Cell Therapy	ALS	Ph III	Enrollment to be completed by mid-2019
Cellerant	romyelocel-L	Cell therapy	bacterial and fungal infections in AML patients	Ph III	To be launched in 2019
Cytori	ECCI-50	Cell therapy	Male stress urinary incontinence	Ph III	Data anticipated in 1H 2019
Fibrocell Science	FCX-007	Gene Therapy	Recessive Dystrophic Epidermolysis Bullosa (RDEB)	Ph III	Expect to initiate Ph3 trial in 1H 2019
GenSight Biologics	GS010	AAV-vector Gene Therapy	Leber Hereditary Optic Neuropathy	Ph III (REVERSE & RESCUE)	Topline data for RESCUE expected Q1 2019; filing for market authorization expected in Europe in Q4 2019 and in the U.S. in H2 2020
Krystal Biotech	KB103	Gene therapy	Dystrophic EB	Ph III	Study anticipated to begin 2H 2019
Mesoblast	Revascor	Allogeneic Mesenchymal Precursor Cell Therapy	Mod to Severe Chronic Heart Failure	Ph III	Complete enrollment Q12019

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Alliance for Regenerative Medicine

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Nightstar Therapeutics	NSR-REP1	Gene therapy	Choroideremia	Ph III	Complete enrollment 1H 2019; data anticipated 2020
Pluristem	PLX-PAD	Cell therapy	Critical limb ischemia	Ph III	H2 2020
Pluristem	PLX-PAD	Cell therapy	Muscle regeneration following hip fracture	Ph III	H2 2020
uniQure	AMT-061	AAV Gene Therapy	Hemophilia B	Pivotal	Dosing of patients expected to start early in the first quarter of 2019
Athersys-Healios KK	MultiStem	Cell therapy	Ischemic Stroke (Japan)	Ph II/III	Ongoing
Atara Biotherapeutics	ATA190	Cell therapy	progressive MS	Ph II	Study to initiate in 2019
Athersys	MultiStem	Cell therapy	Myocardial Infarction	Ph II	Ongoing
Axovant	AXO-Lenti-PD	Gene therapy	Parkinson’s disease	Ph II	Initial data expected March 2019
BioCardia	CardiALLO cell therapy system	Allogenic bone marrow derived cell therapy	Heart failure following heart attack	Ph II	IND approval Q2 2019
BioCardia	CardiAMP cell therapy system	Autologous bone marrow derived cell therapy	Chronic myocardial ischemia	Ph II	Dosing to begin Q1 2019
Caladrius	CLBS03	Cell therapy	Type 1 Diabetes	Ph II	Top-line data expected in early 2019
Caladrius	CLBS12	Cell therapy	CLI	Ph II	Top-line data expected in 1H 2020

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Alliance for Regenerative Medicine

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Caladrius	CLBS14	Cell therapy	CMD	Ph II	Top-line data expected 2H 2019
CytoSen	CSTD002-NK	NK cell therapy	AML	Ph II	Trial to commence 1H 2019
Hemostemix	ACP-01	Cell therapy	critical limb ischemia	Ph II	Interim data by mid 2019
Humacyte	Humacyl	Tissue engineered product	Vascular trauma	Ph II	Topline data expected 2020
Magenta Therpaeutics	MGTA-456	Gene therapy	sickle cell disease	Ph II	Trial to initiate in 1H 2019
Mesoblast	MPC-25-IC	Allogeneic Mesenchymal Precursor Cell Therapy	Acute Myocardial Infarction	Ph II	Data read-out 1H2019
Nohla Tx	Dilanubicel	hematopoietic stem and progenitor cell product	AML patients undergoing intensive chemotherapy	Ph II	2H 2019
Noveome Biotherapeutics, Inc.	ST266	Cell derived secretome therapy	Persistent corneal defects	Ph II	Q2 2019 top line readout
Noveome Biotherapeutics, Inc.	ST266	Cell derived secretome therapy	Cataract surgery	Ph II	Initiating Phase 3 trial Q4 2019, Full results Q4 2020
Poseida Therapeutics	P-BCMA-101	CAR-T therapy	relapsed/refractory multiple myeloma	Ph II	Trial to begin 1H 2019; expected BLA filing by end of 2020
REGENXBIO	RGX-314	Gene therapy	Wet Age-Related Macular Degeneration (wet AMD)	Ph II	Trial expected to initiate in 2019

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Alliance for Regenerative Medicine

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SCM Lifescience	SCM-CGH	Stem cell product (intravenous injection)	Chronic GvHD	Ph II	Dec. 2021
TikoMed AB	IBsolvMIR	Islet Transplantation	Brittle Diabetes	Ph II	Q4 2020
Ziopharm Oncology	Controlled IL-13 + Libtayo	Gene therapy in combination with Libtayo, a PD1 inhibitor	recurrent glioblastoma	Ph II	Trial to initiate in 1H 2019
ReNeuron	CTX product candidate	Cell therapy	Stroke disability	Ph IIb	Ongoing; top-line data expected in early 2020
Bone Therapeutics	ALLOB	Cell therapy (allogeneic)	Spinal Fusion	Ph IIa	efficacy & safety data expected mid 2019
WindMIL Therapeutics	Marrow Infiltrating Lymphocytes (MILs)	Autologous cell therapy	Metastatic NSCLC	Ph IIa	IND clearance – March 2019
WindMIL Therapeutics	Marrow Infiltrating Lymphocytes (MILs)	Autologous cell therapy	Metastatic NSCLC	Ph IIa	Begin enrollment – July 2019
WindMIL Therapeutics	Marrow Infiltrating Lymphocytes (MILs)	Autologous cell therapy	Metastatic NSCLC	Ph IIa	IND clearance – March 2019; Begin enrollment – July 2019
AGTC	ACHM CNGB3 product	Gene therapy	Achromatopsia associated with CNGB3	Ph I/II	Dose escalation completed in Q1 2019
AGTC	XLRP product	Gene therapy	X-Linked Retinitis Pigmentosa	Ph I/II	Dose escalation completed in Q1 2019
Audentes Therapeutics	AT342	Gene therapy	Crigler-Najjar Syndrome	Ph I/II	Next interim data update expected in Q1 2019

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AVROBIO	AVR-RD-01	Gene therapy	Fabry disease	Ph I/II	Update expected Q1 2019
AVROBIO	AVR-RD-02	Gene therapy	Gaucher disease	Ph I/II	Begin to dose patients in 2019
AVROBIO	AVR-RD-04	Gene therapy	Cystinosis	Ph I/II	First patient to be dosed in 2019
Enochian Biosciences	ENO-5001	Genetically modified cell therapy	Oncology	Ph I/II	Q2 2010
Enochian Biosciences	ENO-1001	Genetically modified cell therapy	HIV/AIDS	Ph I/II	Q4 2019
Fibrocell Science	FCX-007	Gene Therapy	Recessive Dystrophic Epidermolysis Bullosa (RDEB)	Ph I/II	Interim data readout and trial update expected in Q1 2019
Genethon	GNT0003	Gene therapy	Crigler-Najjar Syndrome	Ph I/II	Dose escalation completed in 2019
Genethon	GNT001	Gene therapy	Wiskott Aldrich Syndrome	Ph I/II	Completion by end of 2019
Genethon	GNT002	Gene therapy	Chronic Granulomatous Disease	Ph I/II	Treatment phase completed by end of 2019
GenSight Biologics	GS030	Gene therapy	Retinitis Pigmentosa	Ph I/II	Early findings expected 1H 2019; topline results expected 4Q 2020
Homology	HMI-102	Gene therapy	Phenylketonuria	Ph I/II	Ph I/II trial expected to begin and report initial clinical data in 2019
Krystal Biotech	KB103	Gene therapy	Dystrophic EB	Ph I/II	Study anticipated to be completed in 1H 2019

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Medigene	DC Vaccine	Dendritic cell vaccines	AML	Ph I/II	Full data from the trial expected by EOY 2019
REGENXBIO	RGX-111	Gene therapy	MPS I	Ph I/II	Dosing of the first subject expected inthe first half of 2019
REGENXBIO	RGX-121	Gene therapy	MPS II	Ph I/II	Next program updates expected in 2019
REGENXBIO	RGX-501	Gene Therapy	homozygous familial hypercholesterolemia (HoFH)	Ph I/II	Next program updates expected in early 2019
ReNeuron	hRPC product candidate	Cell therapy	Retinitis pigmentosa	Ph I/II	Ongoing; top-line data expected mid-2019
Rocket Pharma	RP-L102	Gene therapy	Fanconi Anemia	Ph I/II	Clinical trial to initiate in 2019
SCM Lifescience	SCM-AGH	Stem cell product (intravenous injection)	Acute GVHD	Ph I/II	July 2021
Sentien Biotechnologies	SBI-101	MSC Device	Acute Kidney Injury	Ph I/II	Q1 2019
Solid Biosciences	SGT-001	AAV-mediated microdystrophin gene transfer	Duchenne muscular dystrophy	Ph I/II	Preliminary results expected in Q1 2019; interim analysis expected 2H 2019
Triumvira	CD19	Adoptive cell therapy	Liquidtumors / CD19+ Malignancies	Ph I/II	Trial to initiate in 1H 2019
Ultragenyx	DTX301	Gene therapy	Ornithine Transcarbamylase (OTC) Deficiency	Ph I/II	Data from Cohort 3 expected mid 2019
ViaCyte	PEC-Direct	Cell Replacement Therapy	Type 1 Diabetes	Ph I/II	2019 – preliminary safety and efficacy data

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Aegle Therapeutics	AGLE-102	Extracellular vesicle therapy	Burns	Ph I/IIa	Expects to initiate trial in early 2019
Asterias	AST-OPC1	Stem cell therapy	Severe spinal cord injury	Ph I/IIa	12 month results expected in Q1 2019
Athersys	MultiStem	Cell therapy	ARDS	Ph I/IIa exploratory	Enrollment completed, results expected 2019 Q1
Bone Therapeutics	ALLOB	Cell therapy (allogeneic)	Delayed-Union Fractures	Ph I/IIa	Expects to submit the CTA for a Ph I/IIb trial in H2 2019
Glycostem	oNKord	Off-The-Shelf NK cell preparation generated from umbilical cord blood	Acute Myeloid Leukemia	Ph I/IIa	First Patient to be enrolled by Q2 2019
Kadimastem	AstroRx	Astrocyte cell therapy	ALS	Ph I/IIa	Initial data from Cohort A expected mid-2019
Myonexus Therapeutics	MYO-101	Gene therapy	limb girdle muscular dystrophy (LGMD) type 2E	Ph I/IIA	Trial expected to initiate mid 2018, with Cohort 1 60 day biopsy data expected 2019
SCM Lifescience	SCM-AGH	Stem cell product (intravenous injection)	Acute Pancreatitis	Ph I/IIa	June 2021
SCM Lifescience	SCM-AGH	Stem cell product (intravenous injection)	Severe Atopic Dermatitis	Ph I/IIa	October 2020
4D Molecular Tx	4D-110	Gene therapy	Choroideremia	Ph I	Clinical trial to initiate in 2019
Adverum Biotechnologies	*ADVM-022*	Gene therapy	Wet AMD	Ph I	Interim trial update expected Q1 2020
Allogene	ALLO-501	T cell therapy	NHL	Ph I	Expected to begin in 1H 2019

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Atara Biotherapeutics	ATA188	Allogenic cell therapy	progressive MS	Ph I	Initial results expected 1H 2019
Axovant	AXO-AAV-GM1	Gene therapy	GM1 gangliosidosis	Ph I	Expected to initiate trial in 1H 2019, with initial data expected in 2H 2019
Axovant	AXO-AAV-GM2	Gene therapy	GM2 gangliosidosis	Ph I	initial data expected in Q1 2019
bluebird bio	bb2121	Anti-BCMA CAR-T	Multiple myeloma	Ph I (CRB-401 study)	Plan to file for marketing authorization in 2019
Cell Medica	CMD-501	CAR-NKT Therapy	Neuroblastoma, small cell lung cancer, melanoma, & other malignancies	Ph I	Interim clinical data to be presented 2H 2019
Cell Medica	CMD-502	CAR-NKT Therapy	Hematological malignancies	Ph I	First patient to be treated in 2019
Celyad	CYAD-01	CAR-T therapy	r/r AML/MDS	Ph I	Preliminary data expected mid-2019
Celyad	CYAD-01	CAR-T therapy	Hematological Malignancies	Ph I	Preliminary data expected H1 2019
Celyad	CYAD-01	CAR-T therapy	r/r AML	Ph I	Preliminary data expected H1 2019
DiscGenics	IDCT	Cell therapy	Degenerative disc disease	Ph I	Expected to begin enrolling in Japan in the first half of 2019
Eureka Therapeutics	ET140202	T cell therapy	Hepatocellular Carcinoma (HCC/Liver Cancer)	Ph I	Expects to initiate U.S. trial in 2019
Eureka Therapeutics	ET140202	T Cell Therapy	Hepatocellular Carcinoma (HCC/Liver Cancer)	Ph I	Expects to initiate U.S. trial in 2019

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MolMed	CAR T CD44v6	CAR-T	haematological tumors	Pre-Ph I	Ph I trial expected to initiate in Q1 2019
Noveome Biotherapeutics, Inc.	ST266	Cell derived secretome therapy	Optic nerve disease, Brain injury/CTE	Ph I	Q3 2019 top line readout
Pluristem	PLX-R18	Cell therapy	Incomplete Hematopoietic Recovery Following Hematopoietic Cell Transplantation (HCT)	Ph I	H1 2020
Sarepta	NT-3	Gene therapy	Charcot-Marie-Tooth Neuropathy type 1A	Ph I	Plan to initiate trial in 2019
SCM Lifescience	SCM-AGH	Stem cell product (intravenous injection)	Severe Atopic Dermatitis	Ph I	October 2020
Selecta Biosciences, CureCN	SVP-Rapamycin	Gene therapy	Crigler-Najjar Syndrome	Ph I	2H 2019
XyloCor	XC001	Gene therapy	refractory angina	Ph I	Trial to commence in 2019
Ziopharm Oncology	Controlled IL-12	Gene therapy	recurrent glioblastoma	Ph I	Enrollment to be completed Q1 2019
Pfizer	PF-06939926	Gene therapy	Duchenne Muscular Dystrophy	Ph Ib	Dosing began March 2018; early data expected 1H 2019
Fate Therapeutics	FT500	iPSC-derived, allogeneic NK Cell	Multi-dose, multi-cycle combination with Checkpoint Inhibitor for Advanced Solid Tumors	IND submission	Awaiting IND approval
Mustang Bio	MB-102	CAR T therapy	acute myeloid leukemia (AML), blastic plasmacytoid dendritic cell neoplasm (BPDCN) and high-risk myelodysplastic syndrome	IND submission	Ph I/II trial to initiate in 2019

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TxCell / Sangamo	TX200	Gene-modified cell therapy	Prevention of chronic rejection after solid organ transplantation	CTA Application	1H 2019
Abeona	ABO-201	Gene therapy	Juvenile Batten disease	Pre-IND	submission of IND first quarter 2020
Abeona	ABO-202	Gene therapy	Infantile Batten disease	Pre-IND	submission of IND first quarter 2019
Allogene	ALLO-715	T cell therapy	r/r multiple myeloma	Pre-IND	Expect to submit an IND and initiate trial in 2019
American Gene Technologies	AGT103-T	Gene therapy	HIV/AIDS	IND enabling	Planned IND submission 2019
Atara Biotherapeutics	Undisclosed	CAR-T Therapy	AML and B cell malignancies	Pre-IND	IND submissions expected Q4 2019 / Q1 2020
Audentes Tx	AT982	Gene therapy	Pompe Disease	pre-IND	Plan to submit IND in 2019
AveXis / Novartis	AVXS-201	Gene therapy	genetic ALS (SOD1)	Pre-IND	IND submission expected in late 2018/early 2019
AveXis / Novartis	AVXS-201	Gene therapy	Rett syndrome (MECP2)	Pre-IND	IND submission expected in late 2018/early 2019
Axovant	AXO-AAV-OPMD	Gene therapy	Oculopharyngeal muscular dystrophy (OPMD)	Pre-IND	Plan to initiate study in 2H 2019
Bellicum	TBD	CAR-T	Undisclosed	pre-IND	Expects to submit INDs for two product candidates in 2019
BioMarin	TBD	Gene therapy	phenylketonuria (PKU)	Pre-IND	IND submission expected in 2H 2019

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Biostage	Cellspan esophageal implant	Tissue engineered product	Pediatric esophageal atresia	Pre-IND	IND submission expected in mid-2019
Fate Therapeutics	FT516	iPSC-derived, allogeneic NK Cell expressing high-affinity, non-cleavable CD16 (hnCD16)	Multi-dose, multi-cycle combination with Monoclonal Antibody for Hematologic Malignancies	Pre-IND	IND to be submitted in 2018
Fate Therapeutics	FT519	iPSC-derived, allogeneic NK Cell expressing anti-CD19 CAR and hnCD16	Hematologic Malignancies	Pre-IND	IND to be submitted in 2019
Fate Therapeutics	FT538	iPSC-derived, allogeneic NK Cell expressing hnCD16 with CD38 knock-out	Multi-dose, multi-cycle combination with daratumumab for Multiple Myeloma	Pre-IND	IND to be submitted in 2019
Fate Therapeutics	FT819	iPSC-derived, allogeneic T Cell expressing anti-CD19 CAR	Hematologic Malignancies	Pre-IND	IND to be submitted in 2019
Genethon	GNT0004	Gene therapy	Duchenne Muscular Dystrophy	Pre-Ph I	CTA and IND submission in 2019
Genethon	GNT0005	Gene therapy	Limb-Girdle Muscular Dystrophy 2i	Pre-Ph I	Recruitment completed in Natural history study by end of 2019
Intellia	ATTR product	Gene editing product	transthyretin amyloidosis	Pre-IND	IND submission anticipated by EOY 2019
Krystal Biotech	KB105	Gene therapy	Lamella Ichthyosis	Pre-IND	IND submission expected in Q4 2018
Lysogene	LYS-GM101	Gene therapy	GM1 gangliosidosis	Pre-IND	Enrollment 2019
NexImmune, Inc	AIM Adoptive Cellular Therapy	Endogenous multi-antigen specific CD8+ T cells	AML, multiple myeloma	Pre-IND	1H 2019 IND

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PTC Therapeutics	TBD	Gene therapy	Friedreich ataxia	pre-IND	Expected to submit IND in 2019
REGENXBIO	RGX-181	Gene therapy	late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease	Pre-IND	IND submission anticipated in 2019
Rubius	RTX-132	Red cell therapy	phenylketonuria (PKU)	Pre-IND	IND submission expected Q1 2019

Sources: Publicly available information and/or company-provided data
Please note: any product-specific questions should be directed to the therapeutic developer
Last Update: December 18, 2018