BioShares™

BBC Portfolio Summary

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BioShares Biotechnology Clinical Trials Fund (Nasdaq: BBC)

Market Cap Data as of 6/19/2017

Ticker	Name	Market Cap ($mm)	Lead Drug(s)	Disease	Phase	Company Description
ACHN	Achillion Pharma	569	ACH-4471	PNH	Phase 1	Achillion's lead drug ACH-4471 is focused on the body's complement system, which is the body's first line of defense against infection and trauma. Excessive activation of the complement system can cause serious inflammatory diseases. Factor D plays a critical role in amplifying the complement system. ACH-4471 targets Factor D, with the first indication being PNH (paroxysmal nocturnal hemoglobinuria). ACHN also has a program targeting Hepatitis C in Phase 2 licensed to J&J.
			Odalasvir	Hepatitis C	Phase 2	
ADRO	Aduro Biotech	734	CRS-207	Mesothelioma	Phase 2b	Aduro is a cancer-focused immunotherapy company with three distinct platforms. Its lead drug CRS-207 utilizes modified listeria bacteria which Aduro calls LADD technology. In May 2016, CRS-207 failed in a Phase 2 study for pancreatic cancer, though the company is pursuing the drug in mesothelioma, gastric and ovarian cancer. ADU-S100 is being studied in Phase 1 studies in collaboration with Novartis.
			ADU-S100	Multiple Tumors	Phase 1	
ADXS	Advaxis	243	Axalimogene filolisbac	Cervical Cancer	Phase 3	Advaxis is focused on developing treatments that redirect the immune system to attack cancer. Advaxis's technology delivers an army of genetically engineered bacteria that allows the patient's immune system to identify tumor specific antigens that would otherwise not be recognized by the immune system. The company has secured partnerships with Bristol Myers, Amgen and Amgen and has 9 different programs across 5 drugs. Lead drug Axalimogene filolisbac is in Phase 3 for cervical cancer.
			ADXS-PSA	Prostate Cancer	Phase 2	
AGEN	Agenus	337	AGEN1884	CTLA-4 antagonist	Phase 1	Agenus has a portfolio of early stage immuno-oncology drug candidates focused on combination therapy for a variety of cancers. Its Mosquirix malaria vaccine was approved in Europe in partnership with Glaxo but has struggled with efficacy due to the mosquito's ability to mutate.
			AGEN2034	PD-1 antagonist	Phase 1	
AGIO	Agios	2,435	Enasidenib	AML	Phase 3	Agios is a precision medicine company with research efforts in 3 areas: (1) cancer metabolism, (2) rare genetic metabolic disorders and (3) metabolic immuno-oncology. Agios has 2 drugs in Phase 3 for AML, and also has a solid tumor focused drug (AG-881) in Phase 1 in collaboration with Celgene.
			Ivosidenib	AML	Phase 3	
			AG-881	Solid tumors	Phase 1	
			AG-348	PK Deficiency	Phase 2	
AIMT	Aimmune	905	AR101	Peanut Allergy	Phase 3	Aimunne is developing new, desensitizing approaches to treat people with food allergies. Their lead product, AR101, has completed Phase 2 trials to treat peanut allergies by introducing peanut protein in incremental doses to desensitize patients to the allergen.
AKAO	Achaogen	881	Plazomicin	Antibiotic	NDA	Achaogen is a clinical-stage biotechnology company focused on developing antibiotics for the treatment of serious bacterial infections due to antibiotic-resistant bacteria that are immediate public threats, and require urgent and aggressive action. Their lead candidate, Plazomicin, recently succeeded in Phase 3 clinical trials and is being submitted as a treatment for carbapenem-resistant enterobacteriaceae (superbug CRE) to the FDA, in which it lowered mortality by 70% compared to the standard of care.
AKBA	Akebia	558	Valdustat	Anemia / CKD (chronic kidney disease)	Phase 3	Akebia focuses on HIF (hypoxia inducible factor) biology which relates to increased red blood cell production and is naturally involved in conditions such as high altitudes. The company's lead drug Valdustat is partnered with Otsuka in a $765mm deal, critical to funding its current Phase 3 program, and competes with Fibrogen and GSK. Akebia has preclinical partnerships involving HIF biology with J&J and Mitsubishi Tanabe.
ALDR	Alder Biopharma	890	Eptinezumab	Migrane Prevention	Phase 3	Alder's lead product, Eptinezumab (previously ALD-403), is an antibody drug targeting CGRP, a small protein involved in heightened sensitivity to pain in migraine and is in Phase 3. Data from Phase 2 trials showed that approximately 1/3 of patients achieved 100 percent reduction of migraines in any given month.
ALNY	Alnylam	6,894	Patisiran	TTR Amyloidosis	Phase 3	Pioneer and leader in RNAi "silencing RNA", a new class of drugs utilizing short DNA fragments to silence mutated RNA fragments which cause inherited diseases. The company is making good progress to its 2020 guidance of achieving 3 marketed products and having 10 programs in clinical trials.
			Inclisiran	Cholesterol	Phase 3	
			Fitusiran	Hemophilia	Phase 3	

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ANAB	AnaptysBio	474	ANB020	Atopic Dermatitis, Peanut Allergy, Eosinophilic Asthma	Phase 2a	AnaptysBio has developed a unique antibody discovery platform which replicates the natural process of antibody generation. It's lead program, ANB020, is being studied for a variety of immune disorders including eczema, with Phase 2a data in H2 2017. In addition, the company has outlicensed antibody drugs to Tesaro for cancer and to Celgene for psoriasis and inflammation. ANAB's IPO was the first biotech in 2017 and was lead managed by Credit Suisse.
			ANB019	Generalized Pustular Psoriasis	Phase 1	
AQXP	Aquinox Pharma	316	AQX-1125	Bladder Pain Syndrome	Phase 3	Lead candidate, AQX-1125 is being evaluated as a treatment for Bladder Pain Syndrome / Interstitial Cystitis (BPS/IC), a disorder that is estimated to affect between 5 and 12 million women in the US. AQXP-1125 is a SHIP-1 activator that is intended to inhibit the migration of immune cells that have an inflammatory effect in the bladder. Data from the first of two Phase III studies is expected in 2018. The company has sufficient cash on hand through 2019.
ARNA	Arena Pharma	400	Etrasimod	Ulcerative colitis	Phase 2	Arena and its partner Eisai obtained FDA approval for Belviq, a weight loss medication in 2012 but sales have been disappointing and Arena has sold most of its rights on Belviq to Eisa. Currently the company's most promising assets unclude Etrasimod and Ralinepag, each in Phase 2.
			Ralinepag	Pulmonary arterial hypertension	Phase 2	
ARRY	Array Biopharma	1,301	Binimetinib	Cancer (MEK)	Phase 3	Array has large pipeline focused on small molecule drugs for cancer. As of June 2017, Array has seven registration (Phase 2 or 3) studies relating to 6 Array drugs and has partnerships with AstraZeneca, Roche, Loxo Oncology and Cascadian Therapeutics. The most advanced is Binimetinib, a small molecule MEK inhibitor that was invented by Array and is two Phase 3 trials for advanced cancer patients, including those with melanoma and colorectal cancer.
			Encorafenib	Cancer (BRAF)	Phase 3	
			Seulmetinib	Cancer (MEK)	Phase 3	
			Ipatasertib	Cancer (AKT)	Phase 3	
			Filanesib	Multiple Myeloma	Phase 2	
ATRA	Atara Bio	358	ATA129	Epstein Barr	Phase 2	Atara is a cancer-focused immunotherapy company utilizing CTLs, or cytotoxic T-cells, to target and destroy cancerous cells or viruses. The company's CTL therapies can be manufactured "off-the-shelf" as opposed to the personalized manufacturing of CAR-T therapies. The company has 3 programs in clinical trial stage.
			ATA188	Multiple sclerosis	Phase 1	
			ATA230	CMV	Phase 2	
AVXS	AveXis	1,876	AVXS-101	Spinal Muscular Atrophy	Phase 1	AveXis is focused on developing gene therapy for patients with rare and life-threatening neurological genetic diseases. Their lead product, AVXS-101, is in Phase 1 clinical trials for the treatment of spinal muscular atrophy, and has been awarded an orphan drug designation from the FDA.
AXON	Axovant Sciences	2,225	RVT-101	Alzheimer's	Phase 3	Axovant is focused on developing and acquiring compounds to treat forms of dementia, including Alzheimer's. The company's lead drug RVT-101 was purchased from GlaxoSmithKline in December 2014 for only $5 million, and has completed Phase 2 studies with strong safety data, and is now in Phase 3 trials. In April 2017, David Hung joined as CEO of the company - he previously was CEO of Medivation which was acquired by Pfizer in 2016 for $14 billion.
			Nelotanserin	Lewy body dementia	Phase 2	
BCRX	Biocryst	445	BCX-7353	Hereditary Angioedema	Phase 2	Biocryst is focusing on developing small molecule drugs for HAE (hereditary angioedema), an inherited disease that leads to painful attacks resulting in life-threatening swelling of the hands, feet, face and airway. BCX-7353 is Biocryst's lead HAE drug and is in Phase 2 trials but has shown some gastro-intestinal toxicities. A second generation drug is in preclinical studies.
BGNE	BeiGene	1,577	BGB-3111	Cancer (BTK)	Phase 2	Beigene is a global biopharma company focused in cancer with major operations in China and headquarters in Fort Lee, NJ. The company has four different drugs in human clinical trials for a variety of tumor types, both as monotherapy and in combination therapy.
			BGB-A317	Cancer (PD-1)	Phase 1	
			BGB-290	Cancer (PARP)	Phase 1	
BLCM	Bellicum	347	BPX-501	Pediatric Orphan Genetic Diseases	Phase 2	Bellicum Pharmaceuticals is a clinical stage biopharmaceutical company focused on the development of novel cellular immunotherapies for cancers that include hematological and solid tumors, and rare blood disorders. Lead product BPX-501 is a T-cell therapy being evaluated for use following an allogeneic hematopoietic stem cell transplant.
			BP-601	Pancreatic cancer	Phase 1	

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BLUE	Bluebird Bio	4,521	LentiGlobin	Sickle Cell / Beta Thalassemia	Phase 2/3	Bluebird is a leading gene therapy company with lead drug LentiGlobin for sickle cell anemia and beta thalassemia. The company's gene therapy platform utilizes a next generation gene therapy technology and has shown remarkable results for these two inherited disorders. The company also has a CAR-T program partnered with Celgene for various cancers, and in June 2017 at ASCO, the company showed strong data multiple myeloma for bb2121.
			bb2121	Multiple Myeloma	Phase 1/2	
BPMC	Blueprint Medicines	1,521	BLU-285	Gastrointestinal stromal tumors	Phase 1	Blueprint is a cancer focused company focusing on drugs which inhibit kinases, which are important targets for genetically defined subsets of cancer patients. Blueprint believes by identifying specific cancer patients which are likely to respond to targeted therapies, this can increase the likelihood of success of drug development. The company currently has three clinical stage programs.
			BLU-554	Hepato-ceullular carcinoma	Phase 1	
			BLU-667	Various cancers	Phase 1	
CALA	Calithera Biosciences	499	CB-839	Renal cell carcinoma / triple negative breast cancer	Phase 2	Calithera discovers and develops small molecule cancer therapies. Lead program CB-839 is being studied in a Phase 2 in combination with everolimus for renal cell carcinoma and in combination with paclitaxel in a Phase 1b/2 for triple negative breast cancer. CB-1158 is partnered with Incyte for various solid tumors in Phase 1.
			CB-1158	Solid tumors	Phase 1	
CARA	Cara Therapeutics	557	CR-845	Pain	Phase 3	Cara Therapeutics is focused on developing novel chemical entities designed to reduce pain by selectively targeting kappa opioid receptors. CR-845 is an IV administered kappa opioid agonist that has demonstrated significant pain relief and a favorable safety profile in three Phase II clinical trials in patients with acute postoperative pain. CR-845 does not bind to mu opioid receptors, avoiding adverse "central" side effects (i.e addiction) seen in opioid analgesics such as morphine
CLDX	Celldex	290	Glem. Vedotin	Breast Cancer	Phase 2	Celldex is an immuno-oncology company that focuses on developing therapies targeting specific patient populations whose diseases express specific markers. Their lead therapy, Glembatumumab vedotin is a fully-human monoclonal antibody-drug conjugate (ADC) that targets glycoprotein NMB (gpNMB). gpNMB is a protein overexpressed by multiple tumor types, including breast cancer and melanoma. gpNMB has been shown to be associated with the ability of the cancer cell to invade and metastasize and to correlate with reduced time to progression and survival in breast cancer. The gpNMB-targeting antibody, CR011, is linked to a potent cytotoxic, monomethyl auristatin E (MMAE), using Seattle Genetics' proprietary technology. Glembatumumab vedotin is designed to be stable in the
			Varlilumab	Head and neck cancer	Phase 2	
CRBP	Corbus	291	Anabasum	Systemic Sclerosis	Phase 2b	Corbus focuses on rare, chronic and serious inflammatory and fibrotic diseases. Lead product candidate anabasum (aka JBT-101 or Resunab) has demonstrated positive results in two Phase 2 studies, one in diffuse cutaneous systemic sclerosis and one in cystic fibrosis.
CTMX	CytomX	495	CX-072	Cancer (PD-L1)	Phase 1	Cytomx's Probody Platform discovers drugs that selectively activate in the tumor microenvironment while reducing drug activity in healthy tissue and in circulation. The company has partnerships with AbbVie, Bristol Myers, Pfizer, ImmunoGen and MD Anderson. Lead program CX-072 is a PD-L1 targeting compound and is wholly owned by Cytomx.
CYTK	Cytokinetics	669	Tirasemtiv	ALS	Phase 3	Cytokinetics focuses on advancing treatments for diseases that are characterized by compromised muscle function. Tirasemtiv is being developed for people living with ALS and other debilitating diseases associated with muscle weakness and fatigue. Tirasemtiv is currently in a large, international Phase 3 trial with results expected in Q4 2017. Omecamtiv mecarbil is a novel cardiac myosin activator investigated for heart failure in partnership with Amgen. Its first Phase 3 trial is expected to enroll 8,000 patients in 900 sites across 35 countries.
			Omecamtiv mecarbil	Chronic Heart Failure	Phase 3	

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DVAX	Dynavax	373	Heplisav-B	Hepatitis B Vaccine	Phase 3	Dynavax is a clinical-stage biopharmaceutical company with multiple product candidates in development for the prevention of infectious disease, the treatment of autoimmune and inflammatory diseases, and the treatment of cancer. They develop immunotherapies based on cutting-edge Toll-Like Receptor (TLR) biology and its ability to modulate the immune system. Their lead asset, Heplisav-B, is seeking FDA approval as a vaccine for Hepatitis B
			AZD-1419	Asthma	Phase 2	
EPZM	Epizyme	846	Tazemetostat	Non-Hodkins Lymphoma	Phase 2	Epizyme focuses on developing small molecule inhibitors of HMTs, a class of epigenetic enzymes. Their lead candidate, Tazemetostat, is an inhibitor of EZH2 (pill) for the treatment of patients with Non-Hodgkin Lymphoma, including germinal center and non-germinal center diffuse large B-cell lymphoma and follicular lymphoma, as well as genetically-defined solid tumors. The company has initiated a phase II monotherapy study of tazemetostat in patients with relapsed or refractory NHL. Epizyme is developing Pinometostat in collaboration with Celgene for the treatment of different types of Leukemia.
			Pinometostat	MLL/AML	Phase 1	
ESPR	Esperion	781	Bempedoic Acid	Cholesterol	Phase 3	Esperion Therapeutics is focused on creating cholesterol lowering therapies. ETC-1002 is a first-in-class oral ACL inhibitor for the treatment of patients with hypercholesterolemia. The company has initiated a phase III study in patients with hyperlipidemia whose LDL-C is not adequately controlled with low and moderate dose statins. They are now able to run a trial on top of maximally-tolerated statins, even though they had trouble with this in the past. This reduces the regulatory burden upon the company and also the amount of money required to develop the asset through FDA submission.
FGEN	Fibrogen	2,053	Roxadustat	Chronic Kidney Disease	Phase 3	FibroGen is a research-based biotechnology company using its expertise in connective tissue growth factor (CTGF) and hypoxia-inducible factor (HIF) biology to discover, develop, and commercialize novel therapeutics for chronic kidney disease, pulmonary fibrosis, pancreatic cancer, and Duchenne muscular dystrophy (DMD).
			FG-3019	Pulmonary Fibrosis	2	
FOLD	Amicus	1,256	Migalastat	Fabry Disease	NDA	Amicus is seeking global approvals for its lead product candidate, migalastat, a personalized medicine in late-stage development to treat individuals with Fabry disease on the basis of their genetic diagnosis. It has been approved in Europe and is pending FDA approval in the United States. Zorblisa, a product candidate in late-stage development, is a potential first-to-market therapy for the chronic, rare connective tissue disorder Epidermolysis Bullosa (EB)
			Zorblisa	Epidermolysis Bullosa	Phase 3	
FPRX	Five Prime	814	FPA008	PVNS	2	Five Prime Therapeutics' lead product, FPA008, is an antibody that inhibits colony stimulating factor-1 receptor (CSF1R), for oncology and inflammatory indications. Five Prime recently announced a partnership agreement with Bristol-Myers Squibb covering Five Prime's CSF1R antibody program. The Company is also evaluating FP-1039 for non-small cell lung cancer (NSCLC) in collaboration with GSK. FP-1039 is in Phase 1b clinical trials for the treatment of Mesothelioma.
			FP-1039	NSCLC	Phase 1b	
GBT	Global Blood	1,169	GBT440	Sickle Cell Disease	Phase 3	GBT is a clinical-stage biotech company focused on developing treatments for blood-based disorders. GBT440 is an oral, once-daily prophylactic therapy for patients with sickle cell disease (SCD) that succeeded in Phase 1/2 trials, and is now moving to enroll a Pivotal Phase 3 trial.
GERN	Geron	411	Imetelstat	Myelofibrosis	Phase 2	Geron is a biopharmaceutical company focused on the development of Imetelstat in partnership with Johnson & Johnson (Janssen). Imetelstat inhibits the activity of telomerase, which is an enzyme present in most types of cancer that enables tumor cells to replicate indefinitely.
				Myelodysplastic Syndromes	Phase 2/3	
GLYC	GlycoMimetics	361	Rivipansel	Sickle Cell	Phase 3	GlycoMimetics is a clinical-stage biotechnology company that uses novel and proprietary glycobiology technology to develop treatments for diseases, particularly where there is high unmet need. Their lead asset is entering a Phase 3
			GMI-1271	AML	Phase 2	

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IMGN	ImmunoGen	405	Mirvetuxumab		Phase 3	ImmunoGen is focused on changing lives, by using our proven ADC technology to develop novel therapies to provide significant benefits to people with cancer. Their technology is used in Roche's Kadcyla, a very large and successful drug launch. Their lead asset, Mirvetuxumab, is being developed for the treatment of cancer that has a very specific genetic mutation. They also have 15 other novel drugs in Phase 1 and 2 clinical trials.
IMMU	Immunomedics	850	Epratuzumab	Acute Lymphoblastic Leukemia	Phase 3	Immunomedics is developing epratuzumab, a humanized antibody which targets an antigen found on the surface of certain white blood cells, for the treatment of lupus and leukemia.
INO	Inovio	596	VGX-3100	Cervical Dysplasia	Phase 3	Inovio Pharmaceuticals is conducting a broad range of clinical trials with their DNA-based immunotherapy platform to prevent and/or treat cancers and infectious diseases. Their lead drug, VGX-3100, is being developed as a vaccine against cervical dysplasia. The purpose of VGX-6150-01's Phase 1 trial is to evaluate the safety and tolerability as a second-line agent in the treatment of HCV.
			VGX-6150-01	Hepatitis C	Phase 1	
INSM	Insmed	992	Arikayse	Lung Infections / Cystic Fibrosis	Phase 3	Insmed is developing treatments for rare lung infections and diseases. Current product candidates utilize proprietary technology designed specifically to deliver drugs to the lung. Insmed is currently conducting two Phase 3 trials with Arikayse for lung infections.
			INS1009	Pulmonary arterial hypertension	Phase 1	
			INS1007	Non-CF bronchiectasis	Phase 1	
ITCI	Intra-Cellular Therapies	479	ITI-007	Schizophrenia	NDA	ITI-007 failed their Phase 3 trial for the treatment of Schizophrenia but are still filing an NDA. Current medications available for the treatment of schizophrenia do not adequately address the broad array of symptoms associated with this CNS disorder. In addition, use of these current medications is limited by their substantial side effects. ITI-007 is designed to be effective across a wider range of symptoms, treating both the acute and residual phases of schizophrenia, with improved safety and tolerability. PDE inhibitor technology offers therapeutic potential across a variety of neurological and cardiovascular diseases. The lead compound, ITI-214 has returned favorable safety and tolerability results in Phase I clinical trials, and ITCI has also initiated a Phase I study for ITI-214 in patients with Parkinsons.
			PDE Inhibitor ITI-214	Parkinson's/CNS disorders	Phase 1	
JNCE	Jounce Therapeutics	539	JTX-2011	Oncology	Phase 1/2	Jounce is a leader in personalized medicine and targeted oncology using their Translational Science Platform. Their lead asset, JTX-2011, is being developed for use in solid tumors.
JUNO	Juno Therapeutics	2,568	JCAR017	Non-Hodgkin Lymphoma	Phase 1	Juno Therapeutics is a therapeutic oncology company genetically modifying patient t cells to treat liquid and solid tumors. The Company's lead program, JCAR017, is being studied in a Phase I trial for Non-Hodgkin Lymphoma. JTCR016 targets the WT1 intracellular protein that is overexpressed in AML and NSCLC, and is currently in the recruitment stage of Juno's Phase I/Phase II trials for the treatment of AML and NSCLC. Juno's JCAR018 targets the CD22 protein, which is expressed by most B-cell lymphomas, including NHL and ALL. The Company is recruiting patients for enrollment in Phase I trials in both of these indications.
			JTCR016	Acute Myeloid Leukemia/NSCLC	Phase 1/2	
			JCAR018	Pediatric ALL/NHL	Phase 1	
KITE	Kite Pharma	5,049	axicabtagene ciloleucel (formerly KTE-C19)	Non-Hodgkins Lymphoma	Pivotal / BLA	Kite Pharma is a therapeutic oncology company genetically modifying patient T-cells to treat liquid and solid tumors. The Company's lead program, axicabtagene ciloleucel (formerly KTE-C19), has been submitted to the FDA for approval as a treatment for relapsed/refractory NHL. Kite is pursuing KTE-C19 as a treatment for a variety of additional indications, with 4 of their treatments currently in Phase I studies. Kite is advancing KITE-718 as their first T-cell receptor product candicate in phase I clinical trials for various solid tumor indications.
			KTE-C19	Liquid tumors	Phase 1	
			KITE-718	Solid tumors	Phase 1	

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KPTI	Karyopharm	433	Selinexor	Non-Hodgkins Lymphoma	Phase 3	Karyopharm Therapeutics has a broad pipeline of treatments being developed for various cancers, inflammatory disorders, and viruses. Selinexor has a unique mechanism and is being evaluated in multiple late stage trials in patients with relapsed and/or refractory blood and solid tumor cancers. Karyopharm is conducting a Phase I/II trial for KPT-8602 in patients with relapsed/refractory multiple myeloma, and is evaluating the drug for safety, tolerability, and efficacy.
			KPT-8602	Multiple Myeloma	Phase 1/2	
LBIO	Lion Biotech	412	LN-144	Melanoma	Phase 2	Lion's TIL Technology bolsters a patients' immune response through replication of specific cancer-fighting cells and reinfusion back into the patient. This approach produced a 49% response rate in treating melanoma, and Lion is currently running a second Phase 2 trial for this indication. Lion's TIL LN-145 utilizes the same mechanism as TIL-144 and is currently being evaluated for safety and efficacy in Phsae II studies for the treatment of both cervical carconoma and SCCHN.
			LN-145	Various cancers	Phase 2	
LJPC	La Jolla Pharma	620	LJPC - 501	Catecholamine-Resistant Hypotension	NDA	La Jolla is developing lead product candidate LJPC-501 to regulate blood pressure in life-threatening situations for which current treatments are ineffective. The Company is currently conducting a Phase III trial with LJPC-501 for catecholamine-resistant hypotension, a condition stemming from dangerously low blood pressure. LJPC-401 is a formulation of synthetic human hepcidin. The drug is currently being evaluated for the treatment of iron overload resulting from HH in a pivotal Phase II study. LJPC-30S is an antibiotic currently in development for the treatment of bacterial infection and genetic disorders such as cystic fibrosis and Duchenne muscular dystrophy.
			LJPC - 401	hereditary hemochromatosis	Phase 2 (pivotal)	
			LJPC - 30S	bacterial infections/genetic disorders	Phase 1	
LOXO	Loxo Oncology	2,079	LOXO-101	Multiple Cancer Types	Phase 2	Loxo Oncology is developing cancer therapies that are caused by single genetic abnormalities. Their lead drug, LOXO-101, announced incredible success in their Phase 2 trial for multiple cancer types in patients with TRK fusions. The company may pursue a Phase 3 trial or go straight for FDA approval. Loxo is enrolling patients in a phase I study to evaluate the RET inhibitor LOXO-292 for the treatment of patients with abnormalities in the rearragned during transfection (RET) kinase.
			LOXO-292	RET gene fusions	Phase 1	
LXRX	Lexicon	1,793	Sotagliflozin	Diabetes	NDA	Lexicon announced topline data for their two diabetes trials utilizing Sotagliflozin on June 10th 2017, which hit all primary and secondary endpoints. They are preparing the drug for NDA submissions in the second half of 2017. Xermelo is approved for the treatment of carcinoid syndrome diarrhea, targeting serotonin production.
			Xermelo	carcinoid syndrome diharrea	Approved	
MCRB	Seres Therapeutics	438	SER-109	Recurrent Clostridium Difficile Infection	Phase 2	Seres Therapeutics is developing therapeutics for diseases and infections resulting from alterations in gastrointestinal bacteria composition. The Company is currently conducting a Phase II trial with SER-109 for recurrent clostridium difficile infection in adults who have had 3 or more occurrences in 9 months. Seres is evaluating SER-262, a synthetically-derived and designed microbiome in a Phase 1b study in patients with primary Clostridium difficile infection. SER-287 is being evaluated in a Phase 1b study in patients with UC.
			SER-262	Primary C. *difficle*	Phase 1b	
			SER-287	Ulcerative Colitis (UC)	Phase 1b	
MGNX	Macrogenics	641	Margetuximab	Cancer and autoimmune diseases	Phase 3	MacroGenics is a late-stage biotechnology company developing antibody-based therapies for patients with cancer. The company has a deep pipeline with 8 candidates in clinical trials, including lead antibody margetuximab, which is in Phase 3 for patients with breast cancer. Several partnerships are in place including a $700 M deal with Johnson and Johnson. Macrogenics is evaluating Teplizimab, an anti-CD2 monoclonal antibody, for the prevention or delayed onset of type 1 diabetes in patients at high risk for developing the disease.
			Teplizimab	Diabetes	Phase 2	

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MYOK	Myokardia	450	MYK-461 MYK-491	HCM dilated cardiomyopathy	Phase 2 Phase 1	MyoKardia, Inc. is pioneering a precision medicine approach to discover, develop and commercialize targeted therapies for the treatment of serious and neglected rare cardiovascular diseases. Their lead product, MYK-461, is being evaluated in two Phase 2 trials for Hypertrophic Cardiomyopathy (both inherited and developed). MYK-491 is currently being evaluated in a Phase 1 clinical trial for the treatment of DCM utilizing a mechanism that established
NERV	Minerva Neurosciences	363	MIN-101 MIN-202 MIN-117	Schizophrenia Mood disorders MDD	Phase 3 Phase 2 Phase 2	Minerva is focused on developing treatments for central nervous system (CNS) diseases. Their lead product, MN-101, will be evaluated in a Phase 3 clinical trial as a treatment for negative symptoms associated with schizophrenia. Additional products are being developed for insomnia, major depressive disorder, and Parkinson's disease.
NVAX	Novavax	311	RSV F Matrix-M Adjuvant Technology	Respitory Syncytial Virus Viral and bacterial infections	Phase 3 Phase 1	Novavax is a clinical-stage vaccine company seeking to prevent a broad array of infectious diseases. The company is conducting Phase 3 trials with lead candidate RSV F for patients with RSV infection. RSV causes a similar number of deaths each year as the flu, and there are currently no approved agents, creating an untapped market opportunity of $6-8 billion.
ONCE	Spark Therapeutics	1,901	Voretigene Neparvovec SPK-7001 SPK-9001 SPK-8011	Inherited Retinal Disease inherited Retinal Disease Hemophilia B Hemophilia A	BLA/NDA Phase 1/2 Phase 1/2 Phase 1/2	Spark Therapeutics is developing gene therapies to restore genetic defects and potential cure patients of eye, hematologic, and neurodegenerative diseases. The company's lead candidate voretigene neparvovec successfully improved vision in a Phase 3 clinical trial and filed their BLA in the first half of 2017.
PBYI	Puma Biotechnology	2,972	Neratinib	Breast Cancer	NDA	Puma Biotechnology is a targeted oncology company developing neratinib for cancer. Neratinib blocks two important growth factor receptors to trigger cancer cell death. They expect to receive FDA feedback by their PDUFA date of June 24th.
PRTA	Prothena	2,060	NEOD001 PRX002	Amyloid related disease Parkinson's	Phase 3 Phase 1	Prothena is developing immunotherapies for the treatment of diseases that invovle amyloid or cell adhesion. Its lead product candidate is NEOD001, is a monoclonal antibody for the treatment of AL amyloidosis. Prothena is currenting running a Phase III trial for NEOD001 in the US and a Phase IIb trial in the EU. The Phase IIb trial may allow conditional approval in Europe.
PRTK	Paratek	665	Omadacycline Seracycline	Antibiotic Antibiotic	NDA NDA	Paratek Pharmaceuticals is focused on the discovery and development of next generation antibiotics. Their lead candidate, Omadacycline, has shown efficacy against MRSA and other antibiotic-resistant community bacterium. Seracycline is a once-daily, oral, tetracycline-derived antibiotic with potent anti-inflammatory properties for the potential treatment of acne and rosacea. The Phase III trial has been completed and met 12-week primary efficacy endpoints.
PTCT	PTC Therapeutics	709	Ataluren RG7800	Muscular Dystrophy Spinal muscal dystrophy	NDA Phase 1b/2a	PTC Therapeutics is developing treatments for Duchenne Muscular Dystrophy (DMD) and Cystic Fibrosis (CF). Their lead candidate, Ataluren, is an orally administered protein restoration therapy for DMD and CF patients carrying nonsense mutations. Ataluren first gained conditional approval in the EU in 2014 to treat nonsense mutation DMD in ambulatory patients 5 years and older. PTC is currently seeking regulatory approval for Ataluren in the US.
PTLA	Portola	2,081	Betrixaban AndexXa	Blood Clots Uncontrolled bleeding events	NDA Approved (3/4b)	Portola seeks to discover and develop novel therapies for the treatment of thrombosis and inflammatory indications. Its product candidate, Betrixaban, is an oral anticoagulant that directly inhibits the activity of Factor Xa, an important validated target in the blood coagulation pathway. The Company's Phase III study with Betrixaban is ongoing.
RARE	Ultragenyx	2,703	KRN23 rhGUS	Bone disease MPS-7	Phase 3 Phase 3	Ultragenyx is developing treatments for rare and ultra rare genetic disorders. The Company's lead candidate is KRN23, a fully human monoclonal antibody targeting Fibroblast Growth Factor 23 (FGF23) in development for X-Linked Hypophosphatemia (XLH) and Tumor-induced Osteomalacia (TIO). KRN23 is currently being evaluated in a Phase II trial in pediatric patients and a Phase III study in adults. rhGUS is an approved treatment for Mucopolysaccharidosis 7.

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BBC Portfolio Summary

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BioShares Biotechnology Clinical Trials Fund (Nasdaq: BBC)

Market Cap Data as of 6/19/2017

Ticker	Name	Market Cap ($mm)	Lead Drug(s)	Disease	Phase	Company Description
RETA	Reata Pharma	632	Bardoxolone Methyl	PAH	Phase 3	Reata's mission is to develop novel therapeutics for patients with serious or life-threatening diseases by targeting molecular pathways involved in regulating cellular metabolism and inflammation. Their lead product candidate, bardoxolone methyl, is a member of a class of small molecules called antioxidant inflammation modulators, or AIMs. Bardoxolone methyl is enrolling patients in their Phase 3 clinical development for the treatment of pulmonary arterial hypertension associated with connective tissue disease.
			omaveloxolone	Mitochondrial Myopathies,	Phase 2	
			RTA 901	Orphan neurological indications	Phase 1	
RGNX	Regenxbio	589	RGX-314	Wet AMD	Phase 1	Regenxbio is the world leader in AAV-based gene therapies. Their lead asset, RGX-314, is being developed for the treatment of wet age-related macular degeneration. They expect to have an interim trial readout in the latter part of 2017. Regenxbio is currently enrolling patients with HoFH in a Phase I/II clinical trial of intravenously administered RGX-501 for the treatment of lipoprotein-deficient HoFH patients. RGX-501 has received orphan drug product designation from the FDA.
			RGX-501	Homozygous familial hypercholesterolemia (HoFH)	Phase 1	
RIGL	Rigel	340	Fostamatinib	Immune Thrombocyto-penia	NDA	Rigel Pharmaceuticals, Inc. is a clinical-stage biotechnology company dedicated to the discovery and development of novel, targeted drugs in the therapeutic areas of immunology, oncology and immuno-oncology. Their lead product, Fostamatinib, has recently completed two Phase 3 clinical trials for the treatment of immune thrombocytopenia, and is pursuing approval with the FDA. R428 is an AXL kinase inhibitor in phase 2 clinical trial for the treatment of patients with AML
			R428 (BGB324)	Cancer	Phase 2	
RXDX	Ignyta	554	Entrectinib	Cancer	Phase 2	Ignyta is a targeted oncology company with a large number of product candidates in development for solid tumors. Entrectinib is the Company's lead product for the treatment of cancers harboring genetic alteration in several receptor tyrosine kinases inclduing TrkA, TrkB, TrkC, ROS1 and ALK. in Phase 2 Pivotal Trials.
			RXDX-105	solid tumors	Phase 1b	
			Taladegib	Ovarian Cancer	Phase 1	
SAGE	Sage Therapeutics	3,133	SAGE-547	Epilepsy	Phase 3	Sage Therapeutics is developing therapeutics to treat epilepsy and other central nervous system conditions. Their lead product, Sage 547, is combined with existing epilepsy treatments to be used on super-refractory patients. SAGE 547 will likely be the last line of defense before brain surgery is required. They are also testing the drug for post-partum depression. SAGE-217 is a novel neuroactive steroid in Phase II development for a number of seizure conditions.
			SAGE-217	Epilepsy	Phase 2	
SGMO	Sangamo Therapeutics	572	SB-525	Hemophilia	Phase 1	Sangamo Biosciences is a clinical stage biopharmaceutical company using their gene editing Zinc finger nuclease technology to correct or disrupt mutated genes. Their lead candidate, SB-525, is being investigated as a gene therapy using AAV to treat hemophilia.
			SB-FIX	Hemophilia B	Phase 1/2	
			SB-318	MPS I	Phase 1/2	
			SB-913	MPS II	Phase 1/2	
TGTX	TG Therapeutics	735	TG-1101	CLL & MCL	Phase 3	TG Therapeutics is focused on the development of novel treatments for B-cell malignancies and autoimmune diseases. Their lead candidate, TG-1101, is a monoclonal antibody that targets a unique epitope on the B-lymphocyte CD20 antigen. CD20 is a highly sought after target for B cell lymphoma and leukemia treatments
			TG-1102	Heme Malignancies	Phase 2	
			TG-1103	CLL	Phase 3	
TTPH	Tetraphase	287	Eravacycline	Bacterial infections	Phase 3	Tetraphase Pharmaceuticals is a clinical-stage life science company with a synthetic chemistry technology platform that has the potential to address the global health crisis caused by antibiotic resistance. Eravacycline is their lead product candidate being developed as a broad-spectrum antibiotic for the treatment of life-threatening infections, including those caused by multidrug-resistant (MDR) Gram-negative bacteria
			TP-271	Antibiotic	Phase 1	
			TP-6076	Antibiotic	Phase 1	
VSAR	Versartis	592	Somavartan	Growth Hormone Deficiency	Phase 3	Versartis' mission is focused on developing, manufacturing and commercializing novel therapeutics to improve and transform treatment for patients with endocrine disorders. Their lead asset, Somavartan, is a long-acting form of recombinant growth hormone that will allow bi-monthly injections rather than once per day.

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XLRN	Acceleron	1,171	Luspatercept	Myelodysplastic Syndromes	Phase 3	Acceleron is developing therapeutics that regulate the transforming growth factor beta (TGF-β) superfamily of proteins, which play fundamental roles in the growth and repair of cells and tissues such as red blood cells, bone, and blood vessels. Lead candidate, Luspatercept, is being developed in concert with Celgene initially for the treatment of chronic anemia.
			ACE-083	facioscapulohumeral muscular dystrophy (FSH)	Phase 2	
XNCR	Xencor	948	XmAb5871	Lupus	Phase 2	Xencor is focused on developing antibodies designed to target new biological mechanisms while being more potent, safer and longer lasting than competitors. Their lead compound, XmAB5871, is a humanized monoclonal antibody that targets the CD19 antigen and co-engages CD32b to treat autoimmune diseases. XmAb5871 has a uniquely selective dual targeting mechanism for B cell inhibition.
			XmAb7195	Asthma	Phase 1	
			XmAb14045	Acute Myeloid Leukemia	Phase 1	
ZIOP	Ziopharm	772	Ad-RTS-hil-12	Breast Cancer	Phase 2	Lead candidate, Ad-RTS-hIL-12 is being evaluated as a treatment for locally advanced or metastatic breast cancer following chemotherapy and malignant glioblastoma. The Company is using its novel cell engineering techniques and multigenic gene programs to develop both patient and donor derived adoptive cell therapies based on genetically modified T cells and natural killer cells. The Company is partnered with Intrexon Corporation, and collaborating with MD Anderson Cancer Center for its programs.

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BBP Portfolio Summary

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BioShares Biotechnology Products Fund (Nasdaq: BBP)

Market Cap Data as of 6/19/2017

Ticker	Name	Market Cap ($mm)	Lead Drug(s)	Disease	Phase	Company Description
ACAD	Acadia	3,416	Nuplazid	Parkinson's	Marketed	Acadia is a Central Nervous System focused company with lead drug Nuplazid. They are also studying the same compound for Alzheimers and schizophrenia. Nuplazid is a new chemical entity designed to treat elderly patients
				Alzheimers	Phase 2	
ACOR	Acorda Therapeutics	831	Ampyra	Multiple Sclerosis	Marketed	Acorda's first drug Ampyra is indicated to improve the walking speed in patients and is selling more than $400 million annually. In 2014, Acorda acquired Civitas Therapeutics for $525 million and is focused on developing its late stage drug for Parkinson's disease.
			CVT-301	Parkinson's	Phase 3	
ALXN	Alexion	26,084	Soliris	PNH	Marketed	Alexion is a leader in developing rare and orphan drugs and its first product Soliris, for PNH (paroxysmal nocturnal hemoglobinuria), is selling more than $2.5 billion. In mid-2015, Alexion acquired Synageva for $9 billion and its drug Kanuma received FDA approval in December 2015 to treat lysosomal acid lipase (LAL) defiency.
			Kanuma	LAL	Marketed	
AMGN	Amgen	121,407	Enbrel	Rheumatoid Arthritis	Marketed	One of the earliest biotech IPOs (1980) and dividend payors (>2% yield), Amgen now has a $20 billion sales base with dominant franchises in red blood cell stimulation for anemia (Epogen and Aranesp) and white blood cell stimulation for neutropenia (Neupogen and Neulasta). Also through the 2001 Immunex acquisition for $16 billion, Amgen is a leading player in rheumatoid arthritis with its Enbrel franchise. These legacy drugs are under competitive threat by the Biosimilars industry, but Amgen is also developing its own Biosimilars for non-Amgen products. Newer products include Prolia/Xgeva, Sensipar and Kyprolis. Most recently in 2015, Amgen received FDA approval and launched potential cholesterol lowering blockbuster Evolocumab.
			Epogen	Anemia	Marketed	
			Neupogen	Neutropenia	Marketed	
			Kyprolis	Multiple Myeloma	Marketed	
			Evolocumab	Hyperlipidemia	Marketed	
BIIB	Biogen	55,264	Tecfidera	Multiple Sclerosis (MS)	Marketed	$10 billion of sales; founded in 1980; pioneer and broad product portfolio in multiple sclerosis therapy including latest blockbuster Tecfidera launched in 2013. High risk / high reward pipeline includes Aducanumab in Phase 3 for Alzheimer's and Anti-LINGO-1 in Phase 2 as a potential disease-modifying treatment for MS (remylenation).
			Avonex	MS	Marketed	
			Tysabri	MS	Marketed	
			Aducanumab	Alzheimer's	Phase 3	
BIVV	Bioverativ	6,547	Eloctate	Hemophilia A	Marketed	BioVerativ is an independent company that was spun out of Biogen's hemophilia business. They are committed to to transforming the lives of people with hemophilia and other blood disorders. The company has nearly $1 billion in sales.
			Alprolix	Hemophilia B	Marketed	
BMRN	Biomarin	15,940	Kuvan	Phenylketonuria	Marketed	Biomarin is an innovator and leader treatment of rare, inherited diseases. The company has 5 drugs on the market and its first drugs are enzyme replacement therapies whereby treatments are given to replace missing or mutated enzymes in diseased patients. Biomarin's latest stage pipeline compound is Drisapersen for Duchenne's muscular dystrophy, and faces a challenging road in front of the FDA as the company awaits an approval decision.
			Vimizim	Morquio A syndrome	Marketed	
			Naglazyme	MPS VI	Marketed	
CELG	Celgene	97,728	Revlimid	Multiple Myeloma	Marketed	One of the largest biotechnology companies with a leading franchise in solid tumors and lymphomas/leukemias. Broad marketed drug portfolio including Revlimid which was approved in 2006 to treat multiple myeloma and generates more than $5 billion of annual sales and accounts for nearly 2/3 of total sales. Highly acquisitive, including 2015 purchase of Receptos for $7 billion. 28 partnered products in clinical trials including Juno's CAR-T.
			Pomalyst	Multiple Myeloma	Marketed	
			Abraxane	Solid tumors	Marketed	
CLVS	Clovis Oncology	3,935	Zejula	Ovarian Cancer	Marketed	Clovis is a targeted oncology company developing therapies with companion diagnostics that direct their product candidate to patiients most likely to benefit from the drug. Their lead product, Rucaparib, is in development for patients with ovarian cancer and has received a Breakthrough Therapy designation from the FDA.
ENTA	Enanta	657	Paritaprevir	Hepatitis C	Marketed	Enanta focuses on liver and viral diseases. Its first drug paritaprevir was approved in 2014 and is sold by Abbvie as part of its Viekira Pack triple-combo for Hepatitis C, competing with Gilead's Sovaldi/Harvoni. Enanta receives double digit royalties.
			ABT-493	Hepatitis C	Phase 3	

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EXEL	Exelixis	6,318	Cometriq	MTC	Marketed	Exelixis is a biopharmaceutical company focused on developing and commercializing small molecule therapies with the potential to improve the treatment of cancer. Their first approved product, COMETRIQ, is indicated for the treatment of patients with progressive, metastatic medullary thyroid cancer (MTC). They also have three compounds in clinical testing, including one they are working on with Roche, for other forms of cancer.
GILD	Gilead	84,702	Harvoni / Sovaldi	Hepatitis C	Marketed	Gilead is a global biopharmaceutical company with a wide range of commercial and clinical-stage products for the treatment of HIV/AIDS, liver diseases, cancer, inflammatory and respiratory diseases, and cardiovascular conditions. The Company's hepatitis C treatments, Harvoni and Sovaldi, are greatly curative of the disease and have generated double-digit billion dollar revenues in recent years.
HALO	Halozyme	1,981	Hylenex	Injections	Marketed	Halozyme Therapeutics is a biotechnology company focused on developing and commercializing novel oncology therapies that target the tumor microenvironment. Revenues are currently generated through royalties, collaborations, and Hylenex sales. Halozyme is conducting Phase II studies with PEGPH2 for various types of cancer in combination with chemotherapies and immunotherapies.
ICPT	Intercept	3,147	Obeticholic acid	Primary biliary cirrhosis	Marketed	Intercept is a biopharmaceutical company leveraging their expertise in bile acid chemistry to develop novel therapeutics for chronic liver diseases. Intercept has submitted an NDA for lead candidate, Obeticholic acid, for Primary Biliary Cirrhosis (PBC), and is currently evaluating the drug in a Phase 3 trial in NASH patients.
			Obeticholic acid	NASH	Phase 3	
INCY	Incyte	25,040	Jakafi (ruxolitinib)	Myelofibrosis / Polycythemia Vera	Marketed	Incyte Corporation is a biopharmaceutical company focused on the discovery, development and commercialization of oncology therapies. Jakafi is approved for patients with myelofibrosis and polycythemia vera, and generated $161 million in sales during Q3 of 2015 which represents 65% growth over the same period in 2014. Incyte recently submitted an NDA for Baricitinib for rheumatoid arthritis, which triggered a $35 million payment from Eli Lilly and could lead to a $100 million payment and global sales royalties if approved.
IRWD	Ironwood	2,671	Linzess (linaclotide)	Constipation	Marketed	Ironwood possesses unique knowledge of an enzyme implicated in many gastrointestinal diseases, which led to the approval Linzess (linaclotide) and partnership with Allergan. Linzess is indicated for constipation and generated $117.5 million in sales during Q3 of 2015, a 47% increase over Q3 of 2014. Ironwood is also conducting a Phase II study with IW-9179 for diabetic gastroparesis.
IONS	Ionis	6,462	Kymanro	Homozygous Familial Hypercholesterolemia (HoFH)	Marketed	Ionis utilizes an RNA-targeted drug discovery platform to develop treatments for severe and rare diseases, and has generated a robust pipeline as a result. Ionis had revenues of $232.1 million in the first 9 months of 2015, a result of partnerships with Bayer, Biogen, Roche, and GSK. Ionis plans to develop and commercialize their Phase III asset volanesorsen through their wholly owned subsidiary, Akcea Therapeutics, to treat familial chylomicroneima syndrome familial partial lipodystrophy.
KERX	Keryx	652	Auryxia	Chronic Kidney Disease	Marketed	Keryx Biopharmaceuticals is focused on the acquisition, development and commercialization of pharmaceutical products for the treatment of life-threatening diseases, including cancer and renal disease. AURYXIA is the Company's approved product for the control of serum phosphorous levels in patients with chronic kidney disease (CKD).The drug launched in the US in 2014.
LGND	Ligand	2,431	Promacta	Hepatitis C	Marketed	Ligand's business is focused on developing programs that lead to licensing deals or acquiring royalty revenue-generating assets and coupling them to an efficient, lean corporate cost structure.The Company has assembled one of the largest and most diversified portfolios of current and future royalty-generating assets in the industry. These therapies address the unmet medical needs of patients for a broad spectrum of diseases including thrombocytopenia, multiple myeloma, diabetes, fungal infections, muscle wasting, dyslipidemia, anemia and osteoporosis.

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MDCO	The Medicines Company	2,726	Angiomax	Anticoagulant	Marketed	The Medicines Company develops treatments for acute and intensive care medicine. The Company has three approved products for acute cardiovascular care, four approved products for suregey and perioperative care, and two approved products for infectious disease.
MDVN	Medivation	#N/A	Xtandi	Prostate Cancer	Marketed	Medivation's Xtandi is indicated for the treatment of patients with metastatic castration-resistant prostate cancer (CRPC). The Company's plans on building a portfolio of four-to-six product candidates that have the potential to be in clinical trials within 18 months of acquisition. They intend to partner or sell successful programs to larger companies when applicable.
MNTA	Momenta	1,265	Enoxaparin Sodium Injection	Deep Vein Thrombosis	Marketed	Momenta is a leader in the analysis, characterization, and design of complex pharmaceutical products. The Company's first approved product was a generic version of LOVENOX for the treatment of deep vein thrombosis. In 2015, Momenta received U.S. regulatory approval for GLATOPA, a fully substitutable generic version of daily COPAXONE. Glatopa is the only approved generic drug available to date for the treatment of patients with relapsing forms of multiple sclerosis.
NBIX	Neurocrine	3,995	Ingrezza	Endocrinology and central nervous system	Marketed	Neurocrine Biosciences focuses on neurological and endocrine-based diseases with high unmet medical needs. The company's lead asse, Ingrezza, was approved to treat Tardive Dyskinesia. Their lead clinical candidate Elagolix, is partnered with AbbVie and has completed a successful Phase 3 trial in patients with endometriosis.
NKTR	Nektar	2,853	MOVANTIK	Opioid-induced Constipation	Marketed	Nektar has a proprietary technology for increasing drug stability and is partnered with several pharmaceutical companies to develop internal and external drug candidates. The company received its first approval of an internal drug candidate in 2014, MOVANTIK, and sales for all products in the first 9 months of 2015 were $191 M.
OMER	Omeros	1,077	Omidria	Cataract Surgery Pain	Marketed	Omeros is a Seattle-based biopharmaceutical company commercializing Omidria, a product that reduces post-operative pain after cataract or other eye surgeries, and was approved in June 2014. Additionally, the company has a deep pipeline has twelve more programs designed to treat illnesses both in large populations, and in orphan disease opportunities.
PGNX	Progenics	485	Relistor	Opioid-induced Constipation	Marketed	Progenics developed Relistor, a mu-opioid receptor antagonist for the treatment of opioid induced constipation in patients with advanced illness or chronic pain. Sales have been under pressure and Progenics along with partner Valeant have submitted an NDA for an oral version of the product that may boost patient interest. Progenics also has a pipeline of targeted oncology candidates.
RDUS	Radius Health	1,831	Tymlos	Osteoporsis	Marketed	Radius Health is focussed on developing treatments for osteporosis and other endocrine-mediated diseases. Tymlos is the Company's lead product for osteporosis and launched in early 2017. Radius is also developing RAD1901 for estrogen receptor positive breast cancer.
REGN	Regeneron	50,134	Eylea	Ophthalmology	Marketed	Regeneron is one of the biggest biotech success stories of the 2000's due to the approval of ophthalmology drug Eylea, which works by blocking new blood vessel formation in patients with age-related macular degeneration. In 2015 the company received approval of its next potential blockbuster, Praluent, for cholesterol management, and the product will be co-commercialized with Sanofi. 2014 sales were $2.8 billion.

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RTRX	Retrophin	720	Cholbam	Bile acid disorders	Marketed	Retrophin develops drugs for severe and rare diseases. The compaby has marketed Chenodal since 2009, and recently acquired the rights to, and began distribution of two key drugs - Thiola and Cholbam. The company expects total net product sales of $100 million. The company has a robust pipeline targeting various kidney and neurological indications.
			Thiola	Kidney stone formation		
SGEN	Seattle Genetics	8,831	Adcetris	Hodgkin Lymphoma	Marketed	Seattle Genetics develops antibody-drug conjugates to kill cancer cells. This technology harnesses the targeting ability of monoclonal antibodies to deliver cytotoxic agents directly to cancer cells. Their lead drug, Adcetris, is approved for relapsed Hodgkin lymphoma or relapsed systemic anaplastic large cell lymphoma. It has been recently approved for patients with classical Hodgkin lyphoma patients at high risk of relapse or progression as post-autologous transplant consolidation. Adcetris has generated $161 million in sales for the first three quarters of 2015.
			SGN-CD33A	AML/MDS	Phase 3	
SGYP	Synergy Pharma	929	Trulance	IBS Chronic Idiopathic Constipation	Marketed	Synergy is a biopharmaceutical company focused on the development and commercialization of novel gastrointestinal therapies. Their primary candidate, Plecanatide, was designed to mimic the function of uroguanylin by working locally in the upper GI tract to activiate and regulate fluid movement needed for healthy bowel function.
SRPT	Sarepta	1,831	Exondys	Muscular Dystrophy	Marketed	The cause of Duchenne muscular dystrophy is a mutation or error in the dystrophin gene, an essential protein used in muscle fiber function. Sarepta's Eteplirsen is designed to skip an exon in the dystrophin pre-m RNA to enable the synthesis of a functional, shorter form of the dystrophin protein.
TBPH	Theravance Biopharma	2,069	Vibativ	Antibiotic	Marketed	Theravance Biopharma has commercialized Vibativ, a once-daily dual-mechanism antibiotic approved for certain difficult to treat infections. The drug has been approved to treat infections due to *Staphylococcus aureus* and other Gram-positive bacteria, including MRSA strains. It has also been approved for the treatment of adult patients with complicated skin and skin structure infections caused by Gram-positive bacteria. Vibativ has generated $11 million in sales for the first three quarters of 2015.
TSRO	Tesaro	7,701	VARUBI	Antiemetic	Marketed	TESARO has several candidates in development to treat variuous types of cancer. Their lead candidate, Niraparib, is a PARP inhibitor that is currently being evaluated in Phase 3 trials against ovarian and breast cancer. In September of 2015, The Company's *Varubi* (rolapitant) was approved to treat chemotherapy induced nausea and vomiting.
			NIRAPARIB	Ovarian and Breast Cancer	Phase 3	
UTHR	United Therapeutics	5,885	Remodulin	PAH	Marketed	United Therapeutics has developed and commercialized three approved therapies for PAH (pulmonary arterial hypertension), a disease characterized by increased pressure in the pulmonary arteries. They have acquired a fourth drug for their PAH franchise, Adcirca, from Eli Lilly in 2008.The company has also commercialized Unituxin for pediatric patients with high-risk neuroblastoma. The revenue generated from all 5 products has totaled $1 billion for the first three quarters in 2015.
			Tyvaso	PAH	Marketed	
VNDA	Vanda	626	Hetlioz	Non-24 Hour Sleep Disorder	Marketed	Vanda Pharmaceuticals focuses on developing and commercializing products for the treatment of central nervous system disorders. Their primary product, Hetlioz, is approved for the treatment of Non-24-Hour Sleep-Wake Disorder. Vanda has also aquired Fanapt, a product for the treatment of schizophrenia, from Novartis. The company expects to generate $110 million in sales from both Hetlioz and Fanapt in 2015.
			Fanapt	Schizophrenia	Marketed	
VRTX	Vertex	31,278	Orkambi	Cystic Fibrosis	Marketed	Vertex Pharmaceuticals has developed and commercizalied therapies for Cystic Fibrosis. Kalydeco, the company's first product, was approved for patients with CF 6 years and older who have the G551D mutation in their CFTR gene. Since launch, the drug's label has been expanded to include patients with additional mutations to the CFTR gene. Kalydeco was approved in 2015 for the treatment of patients 6 years and older with several other mutations to the CFTR gene, broading the market for their CF franchise. The company has generated $582 million in sales for both drugs in the first three quarters of 2015.
			Kalydeco	Cystic Fibrosis	Marketed	

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