PharmExplore

Pharmaceutical Pipeline Intelligence

Genetic Medicine

Pipeline Landscape Report

Generated: November 26, 2025

208 Drugs | 78 Companies

Preclinical: 68 • Phase 1/2: 33 • Phase 3: 28

Table of Contents

Report Summary

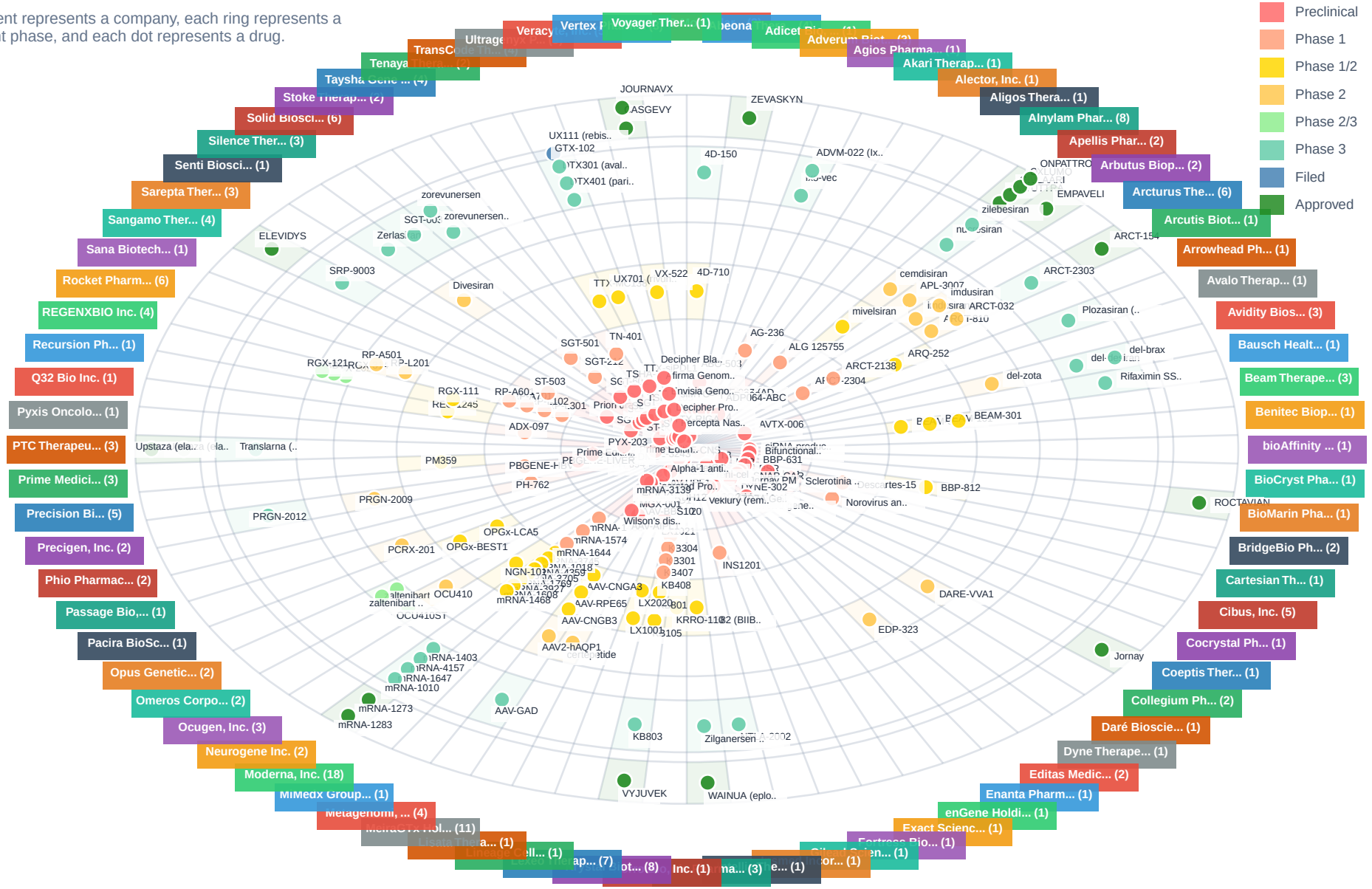
Total Drugs: 208

Companies: 78

Search Criteria: Keywords: gene therapy, editing, RNA in columns: drug_name, indication, therapeutic_area, modality, molecular_target, description

Pipeline Landscape Visualization

Each segment represents a company, each ring represents a development phase, and each dot represents a drug.



Detailed Drug Profiles

4D Molecular Therapeutics, Inc. (3 drugs)

Preclinical

- **4D-725**
  Indication: Alpha-1 Antitrypsin Deficiency
  Therapeutic Area: Pulmonology
  Modality: Gene Therapy

Phase 1/2

- **4D-710**
  Indication: Cystic Fibrosis
  Therapeutic Area: Pulmonology
  Modality: Gene Therapy
  Target: Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Gene
  4D-710 is a genetic medicine that demonstrates successful delivery and expression of the CFTR transgene in the lungs of people with CF.

Phase 3

- **4D-150**
  Indication: Wet Age-Related Macular Degeneration (Wet AMD) And Diabetic Macular Edema (DME)
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Target: vascular endothelial growth factor-C (VEGF-C)
  4D-150 utilizes FDMT's proprietary R100 vector and a transgene cassette encoding aflibercept and inhibitory miRNA targeting VEGF-C, designed to provide multi-year sustained production of anti-VEGF fro...

Abeona Therapeutics Inc. (4 drugs)

Preclinical

- **ABO-504**
  Indication: Stargardt Disease
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Target: ABCA4 Gene
  AAV-based gene therapy for the treatment of Stargardt disease

- **ABO-505**
  Indication: Autosomal Dominant Optic Atrophy (ADOA)
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Target: Opa1 Gene
  AAV-based gene therapy for the treatment of ADOA

- **ABO-503**
  Indication: X-Linked Retinoschisis (XLRS)
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Target: RS1 Protein
  AAV-based gene therapy for the treatment of XLRS

Approved

- **ZEVASKYN**
  Indication: Recessive dystrophic epidermolysis bullosa (RDEB)
  Therapeutic Area: Dermatology
  Modality: Gene therapy
  Gene-modified cellular sheets for the treatment of wounds in adult and pediatric patients with RDEB

Adicet Bio, Inc. (1 drugs)

Preclinical

- **ADI-212**
  Indication: Metastatic Castration-resistant Prostate Cancer (Mcrpc)
  Therapeutic Area: Oncology
  Modality: Gene Therapy
  Target: PSMA

Adverum Biotechnologies, Inc. (3 drugs)

Preclinical

- **BGTF-027 (ADVM-062)**
  Indication: Blue Cone Monochromacy (BCM)
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy

Phase 3

- **Ixo-vec**
  Indication: Wet Age-related Macular Degeneration (Wet Amd)
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Target: Aflibercept
  A single, in-office intravitreal (IVT) injection gene therapy product designed to deliver long-term durable therapeutic levels of aflibercept

- **ADVM-022 (Ixo-vec, previously known as)**
  Indication: Wet age-related macular degeneration (wet amd)
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Target: aflibercept
  A single, in-office intravitreal (IVT) injection gene therapy product designed to deliver long-term durable therapeutic levels of aflibercept

Agios Pharmaceuticals, Inc. (1 drugs)

Phase 1

- **AG-236**
  Indication: PV
  Therapeutic Area: Dermatology
  Modality: SiRNA
  Target: TMPRSS6 gene
  Product candidate for the potential treatment of patients with PV.

Akari Therapeutics, Plc (1 drugs)

Preclinical

- **PHP-303**
  Modality: Small Molecule
  A small molecule selective and reversible neutrophil elastase inhibitor, for which the company intends to seek strategic partners to further its development externally.

Alector, Inc. (1 drugs)

Preclinical

- **ADP064-ABC**
  Indication: Alzheimer's Disease
  Therapeutic Area: Neurology
  Modality: SiRNA
  Target: Tau
  a brain-penetrant anti-tau siRNA paired with Alector Brain Carrier (ABC) technology

Aligos Therapeutics, Inc. (1 drugs)

Phase 1

- **ALG 125755**
  Indication: Chronic HBV Infection
  Therapeutic Area: Infectious Diseases
  Modality: Sirna
  Target: Hbsag
  An siRNA drug candidate targeting HBsAg production

Alnylam Pharmaceuticals, Inc. (8 drugs)

Phase 1/2

- **mivelsiran**
  Indication: Alzheimer's Disease, Cerebral Amyloid Angiopathy
  Therapeutic Area: Neurology
  Modality: Oral Therapeutic
  Target: Amyloid Precursor Protein
  An investigational RNAi therapeutic targeting amyloid precursor protein in development for the treatment of Alzheimer's disease and cerebral amyloid angiopathy.

Phase 2

- **cemdisiran**
  Indication: Complement Mediated Diseases
  Therapeutic Area: Immunology
  Modality: Oral Therapeutic
  Target: C5
  An investigational RNAi therapeutic targeting C5 in development for the treatment of complement-mediated diseases.

Phase 3

- **nucresiran**
  Indication: ATTR Amyloidosis With Cardiomyopathy
  Therapeutic Area: Cardiovascular
  Modality: Oral Therapeutic
  Target: TTR
  An investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis.

- **zilebesiran**
  Indication: Hypertension
  Therapeutic Area: Cardiovascular
  Modality: Oral Therapeutic
  Target: Angiotensinogen
  An investigational RNAi therapeutic targeting angiotensinogen in development for the treatment of hypertension.

Approved

- **AMVUTTRA**
  Indication: ATTR Amyloidosis With Cardiomyopathy, Hereditary Transthyretin-mediated Amyloidosis
  Therapeutic Area: Cardiovascular
  Modality: Oral Therapeutic
  Target: TTR
  An approved RNAi therapeutic for the treatment of ATTR amyloidosis with cardiomyopathy and hereditary transthyretin-mediated amyloidosis.

- **GIVLAARI**
  Indication: Acute Hepatic Porphyria
  Therapeutic Area: Gastroenterology
  Modality: Oral Therapeutic
  Target: Alas1
  An approved RNAi therapeutic for the treatment of acute hepatic porphyria.

- **OXLUMO**
  Indication: Primary Hyperoxaluria Type 1
  Therapeutic Area: Nephrology
  Modality: Oral Therapeutic
  Target: HAO1
  An approved RNAi therapeutic for the treatment of primary hyperoxaluria type 1.

- **ONPATTRO**
  Indication: Hereditary Transthyretin-mediated Amyloidosis
  Therapeutic Area: Neurology
  Modality: Oral Therapeutic
  Target: TTR
  An approved RNAi therapeutic for the treatment of hereditary transthyretin-mediated amyloidosis.

Apellis Pharmaceuticals, Inc. (2 drugs)

Phase 2

- **APL-3007**
  Indication: Geographic Atrophy
  Therapeutic Area: Ophthalmology
  Modality: Sirna
  Target: C3

Approved

- **EMPAVELI**
  Indication: Paroxysmal Nocturnal Hemoglobinuria, C3 Glomerulopathy, Primary Immune Complex Membranoproliferative Glomerulonephritis
  Therapeutic Area: Dermatology
  Modality: Small Molecule
  Target: C3
  Pegcetacoplan, approved by the FDA for the treatment of paroxysmal nocturnal hemoglobinuria, C3 glomerulopathy, and primary immune complex membranoproliferative glomerulonephritis

Arbutus Biopharma Corporation (2 drugs)

Phase 2

- **imdusiran (AB-729)**
  Indication: Chronic Hepatitis B (Chbv) Infection
  Therapeutic Area: Infectious Diseases
  Modality: RNA Interference (RNAi) Therapeutic
  Target: HBV Antigens
  GalNAc-conjugated, subcutaneously-delivered RNAi therapeutic that suppresses all HBV antigens, including HBsAg

- **imdusiran**
  Indication: Chronic hepatitis b (chbv) infection
  Therapeutic Area: Infectious Diseases
  Modality: Oral Therapeutic
  Target: HBV antigens
  a proprietary, conjugated GalNAc, subcutaneously-delivered RNAi therapeutic product candidate that suppresses all HBV antigens, including HBsAg expression

Arcturus Therapeutics Holdings Inc. (6 drugs)

Phase 1

- **ARCT-2304**
  Indication: Pandemic avian influenza (h5n1)
  Therapeutic Area: Infectious Diseases
  Modality: Mrna
  A sa-mRNA vaccine candidate for pandemic avian influenza

- **ARCT-2138**
  Indication: Seasonal influenza
  Therapeutic Area: Infectious Diseases
  Modality: Mrna
  A sa-mRNA seasonal influenza vaccine candidate encoding haemagglutinin (HA) and neuraminidase (NA) of four seasonal influenza strains

Phase 2

- **ARCT-810**
  Indication: Ornithine Transcarbamylase Deficiency
  Therapeutic Area: Rare Diseases
  Modality: MRNA
  An mRNA therapeutic candidate for ornithine transcarbamylase deficiency

- **ARCT-032**
  Indication: Cystic Fibrosis (CF)
  Therapeutic Area: Respiratory
  Modality: Mrna
  an investigational inhaled mRNA therapy for people with cystic fibrosis

Phase 3

- **ARCT-2303**
  Indication: COVID-19
  Therapeutic Area: Infectious Diseases
  Modality: MRNA
  Target: SARS-CoV-2
  A self-amplifying mRNA COVID-19 vaccine

Approved

- **ARCT-154**
  Indication: Covid-19
  Therapeutic Area: Infectious Diseases
  Modality: Mrna
  Target: SARS-CoV-2
  A self-amplifying mRNA COVID-19 vaccine

Arcutis Biotherapeutics, Inc. (1 drugs)

Phase 1/2

- **ARQ-252**
  Indication: Chronic Hand Eczema, Vitiligo
  Therapeutic Area: Dermatology
  Modality: Small Molecule
  Target: JAK1
  ARQ-252 is an alternative topical cream formulation of ivarmacitinib, a potent and highly selective small molecule inhibitor of JAK1, for the treatment of chronic hand eczema and vitiligo.

Arrowhead Pharmaceuticals, Inc. (1 drugs)

Phase 3

- **Plozasiran (ARO-APOC3)**
  Indication: Familial chylomicronemia syndrome (FCS), Severe Hypertriglyceridemia
  Therapeutic Area: Cardiovascular
  Modality: RNAi therapeutic
  Target: Apolipoprotein C-III (apoC-III)
  Designed to reduce production of Apolipoprotein C-III (apoC-III), a component of triglyceride rich lipoproteins

Avalo Therapeutics, Inc. (1 drugs)

Preclinical

- **AVTX-006**
  Modality: Small Molecule
  A non-core asset, a second generation mTORC1/2 inhibitor, for which Avalo is exploring strategic alternatives

Avidity Biosciences, Inc. (3 drugs)

Phase 2

- **del-zota**
  Indication: Duchenne muscular dystrophy (DMD44)
  Therapeutic Area: Rare diseases
  Modality: Antisense oligonucleotide
  Target: Dystrophin mRNA
  Del-zota is designed to deliver phosphorodiamidate morpholino oligomers (PMOs) to skeletal muscle and heart tissue to specifically skip exon 44 of dystrophin mRNA, enabling production of near full-len...

Phase 3

- **del-desiran**
  Indication: Myotonic dystrophy type 1 (dm1)
  Therapeutic Area: Rare Diseases
  Modality: Sirna
  Target: DMPK mRNA
  Del-desiran is designed to address the root cause of DM1 by reducing levels of a disease-related mRNA called DMPK, and consists of a proprietary monoclonal antibody (mAb) conjugated with an siRNA targ...

- **del-brax**
  Indication: Facioscapulohumeral Muscular Dystrophy (FSHD)
  Therapeutic Area: Rare Diseases
  Modality: Sirna
  Target: Dux4 Mrna
  Del-brax is designed to directly target DUX4 in people living with FSHD, and consists of a proprietary mAb conjugated with an siRNA targeting DUX4 mRNA.

Bausch Health Companies Inc. (1 drugs)

Phase 3

- **Rifaximin SSD formulation**
  Indication: Prevention Of OHE And Other Complications In Patients With Early Decompensation In Liver Cirrhosis (RED-C)
  Therapeutic Area: Gastroenterology
  Modality: Small Molecule
  Target: Beta-subunit Of Bacterial Dna-dependent Ribonucleic Acid (RNA) Polymerase
  A next-generation rifaximin formulation that acts by targeting the beta-subunit of bacterial DNA-dependent ribonucleic acid (RNA) polymerase.

Beam Therapeutics Inc. (3 drugs)

Phase 1/2

- **BEAM-302**
  Indication: AATD
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: E342k Point Mutation
  BEAM-302 is a base editing treatment for AATD, designed to correct the disease-causing mutation and restore AAT physiology.

- **BEAM-101**
  Indication: Sickle Cell Disease (SCD)
  Therapeutic Area: Dermatology
  Modality: Gene Therapy
  No new description available

- **BEAM-301**
  Indication: Gsdia
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: R83c Mutation
  BEAM-301 is a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation for GSDIa.

Benitec Biopharma Inc. (1 drugs)

BioCryst Pharmaceuticals, Inc. (1 drugs)

Preclinical

- **Bifunctional Complement Inhibitor**
  Indication: Complement Mediated Diseases
  Therapeutic Area: Rare Diseases
  Modality: Monoclonal Antibody
  Target: C2
  The company is developing a bifunctional complement inhibitor anti-C2 monoclonal antibody that could be a first-in-class combined inhibitor of the classical, lectin and alternative pathways of the com...

BioMarin Pharmaceutical Inc. (1 drugs)

Approved

- **ROCTAVIAN**
  Indication: Severe Hemophilia A
  Therapeutic Area: Dermatology
  Modality: Gene Therapy
  Roctavian is used to treat severe Hemophilia A, a rare genetic disorder.

BridgeBio Pharma, Inc. (2 drugs)

Preclinical

- **BBP-631**
  Indication: Congenital adrenal hyperplasia
  Therapeutic Area: Endocrinology
  Modality: Gene Therapy

Phase 1/2

- **BBP-812**
  Indication: Canavan Disease
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  BBP-812 is an intravenous AAV9 investigational drug product intended for the treatment of children with Canavan Disease.

Cartesian Therapeutics, Inc. (1 drugs)

Phase 1

- **Descartes-15**
  Indication: Multiple Myeloma
  Therapeutic Area: Oncology
  Modality: MRNA CAR-T
  Target: BCMA
  A next-generation, autologous anti-BCMA mRNA CAR-T designed to be more resistant to recycling of the CAR upon multiple antigen exposures.

Cibus, Inc. (5 drugs)

Preclinical

- **HT3**
  Indication: Herbicide Tolerance
  Therapeutic Area: Agriculture
  Modality: Gene Editing
  A productivity trait for Rice

- **HT2**
  Indication: Herbicide Tolerance
  Therapeutic Area: Agriculture
  Modality: Gene Editing
  A productivity trait for Canola

- **PSR**
  Indication: Pod shatter reduction
  Therapeutic Area: Agriculture
  Modality: Gene Editing
  A productivity trait for Canola and Winter Oilseed Rape (WOSR)

- **HT1**
  Indication: Herbicide Tolerance
  Therapeutic Area: Agriculture
  Modality: Gene Editing
  A productivity trait for Rice

- **Sclerotinia resistance**
  Indication: Sclerotinia Resistance
  Therapeutic Area: Agriculture
  Modality: Gene Editing
  A productivity trait for Canola and Soybean

Cocrystal Pharma, Inc. (1 drugs)

Phase 1

- **Norovirus antiviral candidate**
  Indication: Norovirus Infection
  Therapeutic Area: Infectious Diseases
  Modality: Small Molecule
  Target: Rna-dependent RNA Polymerase And Protease
  A novel antiviral candidate for the prophylactic and therapeutic treatment of norovirus infection.

Coeptis Therapeutics Holdings, Inc. (1 drugs)

Preclinical

- **SNAP-CAR**
  Indication: Solid Tumors, Breast Cancer, Ovarian Cancer
  Therapeutic Area: Oncology
  Modality: Gene Therapy
  A technology platform for use with CAR T-cells and NK cells.

Collegium Pharmaceutical, Inc. (2 drugs)

Preclinical

- **Jornay PM**
  Therapeutic Area: ADHD
  A methylphenidate product for the treatment of ADHD.

Approved

- **Jornay**
  Indication: Attention deficit hyperactivity disorder (adhd)
  Therapeutic Area: Neurology
  Modality: Small Molecule
  A central nervous system (CNS) stimulant prescription medicine that contains methylphenidate HCl, a Schedule II methylphenidate.

Daré Bioscience, Inc. (1 drugs)

Phase 2

- **DARE-VVA1**
  Indication: Dyspareunia
  Therapeutic Area: Reproductive Health
  investigational formulation of tamoxifen in a soft gelatin capsule for intravaginal administration as a hormone-free alternative to estrogen-based therapies

Dyne Therapeutics, Inc. (1 drugs)

Preclinical

- **DYNE-302**
  Indication: FSHD
  Therapeutic Area: Neurology
  Modality: MRNA Therapy
  Target: Dux4
  DYNE-302 is being developed to treat Facioscapulohumeral muscular dystrophy (FSHD) by reducing DUX4 expression in muscle tissue.

Editas Medicine, Inc. (2 drugs)

Preclinical

- **reni-cel**
  Indication: Sickle Cell Disease (SCD) And Transfusion-Dependent Beta Thalassemia (TDT)
  Therapeutic Area: Dermatology
  Modality: Gene Therapy
  an experimental ex vivo gene-edited medicine

- **in vivo gene editing medicines**
  Indication: Liver Diseases
  Therapeutic Area: Gastroenterology
  Modality: Gene Therapy
  using in vivo CRISPR editing to upregulate target protein expression and reduce a disease-associated biomarker

Enanta Pharmaceuticals, Inc. (1 drugs)

Phase 2

- **EDP-323**
  Indication: RSV
  Therapeutic Area: Infectious Diseases
  Modality: Small Molecule
  Target: N-Protein
  An oral, direct-acting antiviral selectively targeting the L-protein, a viral RNA-dependent RNA polymerase enzyme that contains multiple enzymatic activities required for RSV replication.

Exact Sciences Corporation (1 drugs)

Preclinical

- **CRC blood test**
  Indication: Colorectal Cancer
  Therapeutic Area: Oncology
  Modality: Other
  An internal version of a blood-based screening test for colorectal cancer, showing sensitivities of 73% for CRC and 14% for advanced precancerous lesions at 90% specificity.

Fortress Biotech, Inc. (1 drugs)

Preclinical

- **AAV-ATP7A Gene Therapy**
  Indication: Menkes Disease
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  AAV-ATP7A gene therapy has demonstrated the ability to rescue neurological phenotypes and improve survival when coadministered with copper histidinate injections in a mouse model of Menkes disease

Gilead Sciences, Inc. (1 drugs)

Preclinical

- **Veklury (remdesivir)**
  Indication: COVID-19
  Therapeutic Area: Infectious Diseases
  Modality: Small Molecule
  A nucleotide analog RNA polymerase inhibitor for the treatment of COVID-19

Insmed Incorporated (1 drugs)

Phase 1

- **INS1201**
  Indication: Dmd
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  An intrathecally delivered gene therapy for the treatment of DMD

Intellia Therapeutics, Inc. (1 drugs)

Phase 3

- **NTLA-2002**
  Indication: Hereditary Angioedema (HAE)
  Therapeutic Area: Immunology
  Modality: Gene therapy
  Target: KLKB1 gene
  NTLA-2002 is a wholly owned, investigational in vivo CRISPR-based therapy designed to knock out the KLKB1 gene in the liver, with the goal of achieving lifelong control of HAE attacks after a single d...

Ionis Pharmaceuticals, Inc. (3 drugs)

Phase 1/2

- **ION582 (BIIB121)**
  Indication: Angelman Syndrome
  Therapeutic Area: Neurology
  Modality: Antisense Oligonucleotide
  Target: UBE3A Antisense Transcript (UBE3A-ATS)
  ION582 is an investigational RNA-targeted medicine designed to inhibit the expression of UBE3A-ATS and increase production of UBE3A protein, for the potential treatment of AS.

Phase 3

- **Zilganersen (formerly ION373)**
  Indication: Alexander Disease (AxD)
  Therapeutic Area: Neurology
  Modality: Antisense Oligonucleotide
  Target: Glial Fibrillary Acidic Protein (GFAP)
  Zilganersen is an investigational RNA-targeted medicine designed to inhibit the production of GFAP, which accumulates due to disease-causing variants in the GFAP gene.

Approved

- **WAINUA (eplontersen)**
  Indication: Hereditary Transthyretin-mediated Amyloidosis (Attrv-pn)
  Therapeutic Area: Neurology
  Modality: Antisense Oligonucleotide
  Target: Transthyretin (TTR)
  WAINUA causes degradation of mutant and wild-type TTR mRNA through binding to the TTR mRNA, resulting in a reduction of serum TTR protein and TTR protein deposits in tissues.

Korro Bio, Inc. (1 drugs)

Phase 1/2

- **KRRO-110**
  Indication: AATD
  Therapeutic Area: Rare Diseases
  Modality: Oligonucleotide
  Target: SERPINA1
  KRRO-110 is a treatment for AATD using a proprietary RNA editing approach to repair a pathogenic variant on RNA.

Krystal Biotech, Inc. (8 drugs)

Phase 1

- **KB304**
  Indication: Wrinkles of the décolleté
  Therapeutic Area: Dermatology
  Modality: Gene Therapy
  Target: COL3A1, ELN
  KB304 is a solution formulation of a novel vector for intradermal injection designed to deliver two copies of the COL3A1 transgene and one copy of the ELN transgene to address various signs of skin ag...

- **KB301**
  Indication: Lateral Canthal Lines At Rest And Dynamic Wrinkles Of The Décolleté
  Therapeutic Area: Dermatology
  Modality: Gene Therapy
  Target: Col7a1
  KB301 is a solution formulation of a novel vector for intradermal injection designed to deliver two copies of the COL3A1 transgene to address signs of aging or damaged skin caused by declining levels ...

- **KB407**
  Indication: Cystic Fibrosis
  Therapeutic Area: Respiratory
  Modality: Gene Therapy
  Target: CFTR
  KB407 is an inhaled (nebulized) formulation of a novel vector designed to deliver two copies of the full-length cystic fibrosis transmembrane conductance regulator (CFTR) transgene for the treatment o...

- **KB408**
  Indication: Alpha-1 Antitrypsin Deficiency (AATD) Lung Disease
  Therapeutic Area: Respiratory
  Modality: Gene Therapy
  Target: Serpina1
  KB408 is an inhaled (nebulized) formulation of a novel vector designed to deliver two copies of the SERPINA1 transgene, encoding for normal human alpha-1 antitrypsin (AAT) protein, for the treatment o...

Phase 1/2

- **KB801**

  Indication: Neurotrophic Keratitis (NK)

  Therapeutic Area: Ophthalmology

  Modality: Gene Therapy

  KB801 is an eye drop formulation of a novel HSV-1 based vector designed to deliver two transgene copies to the corneal epithelium for the sustained, localized expression and secretion of nerve growth ...

- **KB105**

  Indication: Lamellar Ichthyosis (LI)

  Therapeutic Area: Dermatology

  Modality: Gene therapy

  Target: TGM1

  KB105 is a topical gel containing a novel vector designed to deliver two copies of the TGM1 transgene encoding the human enzyme transglutaminase-1 (TGM1) for the treatment of LI, a serious rare skin d...

Phase 3

- **KB803**

  Indication: Ocular Complications In Patients With DEB

  Therapeutic Area: Ophthalmology

  Modality: Gene Therapy

  KB803 is a redosable eye drop formulation of B-VEC, designed for the treatment of ocular complications that are thought to affect over 25% of DEB patients.

Approved

- **VYJUVEK**

  Indication: Dystrophic Epidermolysis Bullosa (DEB) patients from birth

  Therapeutic Area: Dermatology

  Modality: Gene Therapy

  Target: Col7a1

  VYJUVEK is a non-invasive, topical, redosable gene therapy approved in the United States, Europe, and Japan for the treatment of DEB, a rare and severe monogenic disease that affects the skin and muco...

Lexeo Therapeutics, Inc. (7 drugs)

Preclinical

- **LX2021**
  Indication: Desmoplakin Cardiomyopathy
  Therapeutic Area: Cardiovascular
  Modality: Gene Therapy
  Target: Cx43
  Gene therapy candidate designed to intravenously deliver a functional Cx43 gene to myocardial cells for the treatment of Desmoplakin cardiomyopathy.

- **LX2022**
  Indication: Hypertrophic cardiomyopathy
  Therapeutic Area: Cardiovascular
  Modality: Gene Therapy
  Target: TNNI3
  Gene therapy candidate designed to intravenously deliver a functional TNNI3 gene to myocardial cells to treat a distinct form of hypertrophic cardiomyopathy due to mutations in the TNNI3 gene.

- **LX1020**
  Indication: Alzheimer's disease
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Target: APOE2
  Early-stage AAVrh10-based gene therapy candidate designed to express the protective APOE2 protein in the CNS of APOE4 homozygous patients, while concurrently delivering miRNA to suppress the expressio...

- **LX1021**
  Indication: Alzheimer's Disease
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Target: APOE2
  Early-stage AAVrh10-based gene therapy candidate designed to express the Christchurch-modified APOE2 protein in the CNS of APOE4 homozygous patients.

Phase 1/2

- **LX2020**
  Indication: Pkp2-acm
  Therapeutic Area: Cardiovascular
  Modality: Gene Therapy
  Target: Pkp2
  AAVrh10-based gene therapy candidate designed to intravenously deliver a functional PKP2 gene to cardiac muscle for the treatment of PKP2-ACM.

- **LX1001**
  Indication: Alzheimer's Disease
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Target: Apoe2
  AAVrh10-based gene therapy candidate designed to express the protective APOE2 protein in the CNS of APOE4 homozygous patients.

Lineage Cell Therapeutics, Inc. (1 drugs)

Preclinical

- **RND1**
  Indication: Undisclosed Indication
  Modality: Car T Cell Therapy
  A cell transplant program, in development through a gene editing collaboration with Factor Biosciences Limited

Lisata Therapeutics, Inc. (1 drugs)

Phase 2

- **certepetide**
  Indication: solid tumors, metastatic pancreatic ductal adenocarcinoma
  Therapeutic Area: Oncology
  Modality: Peptide
  Target: alpha-v beta-3 and alpha-v beta-5 integrins
  Certepetide is a nine amino acid cyclic proprietary internalizing RGD (iRGD) peptide that activates the C-end rule (CendR) active transport mechanism, allowing co-administered anti-cancer drugs to tar...

MeiraGTx Holdings plc (11 drugs)

Preclinical

- **AAV-RetGC**
  Indication: Leber Congenital Amaurosis Type 1 Due To GUCY2D Mutations
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Gene therapy product candidate to treat LCA1 due to GUCY2D mutations

- **AAV-UPF1**
  Indication: Amyotrophic Lateral Sclerosis
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Viral vector product candidate to increase UPF1 expression in the motor neurons of ALS patients

- **AAV-RDH12**
  Indication: RDH12-Associated Retinal Dystrophy
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  AAV-based gene therapy designed to deliver a functional copy of the RDH12 gene to the retina of patients with genetically defined RDH12 deficiency

- **AAV-BBS10**
  Indication: Bardet-biedl Syndrome Due To Bbs10 Mutations
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Adeno-associated virus with a serotype 8 capsid with a complementary DNA (cDNA) encoding the human BBS10 gene for treatment of BBS due to BBS10 mutations

- **AAV-AIPL1**
  Indication: Lca4
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  AAV-AIPL1 is a gene therapy product candidate for the treatment of LCA4.

Phase 1/2

- **AAV-CNGA3**
  Indication: Achromatopsia Related To Mutations In CNGA3 Gene
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Gene therapy product candidate for the treatment of achromatopsia

- **AAV-RPE65**
  Indication: Retinal Dystrophy Related To Mutations In Rpe65 Gene
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Gene therapy product candidate for the treatment of retinal dystrophy

- **AAV-CNGB3**
  Indication: Achromatopsia Related To Mutations In CNGA3 Gene
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Gene therapy product candidate for the treatment of achromatopsia

Phase 2

- **AAV2-hAQP1**
  Indication: Radiation-Induced Xerostomia
  Therapeutic Area: Oncology
  Modality: Gene Therapy
  AAV2-hAQP1 is a gene therapy product candidate for the treatment of radiation-induced xerostomia.

Phase 3

- **AAV-GAD**
  Indication: Parkinson's Disease
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  AAV-GAD is a gene therapy product candidate for the treatment of Parkinson's disease.

Metagenomi, Inc. (4 drugs)

Preclinical

- **Alpha-1 antitrypsin deficiency program**
  Indication: Alpha-1 Antitrypsin Deficiency
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: Piz
  Gene editing therapy for alpha-1 antitrypsin deficiency

- **Secreted Protein Deficiencies program**
  Indication: Secreted protein deficiencies
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Therapeutic candidates targeting secreted protein deficiencies

- **MGX-001**
  Indication: Severe Hemophilia A
  Therapeutic Area: Dermatology
  Modality: Gene Therapy
  Target: FVIII
  Gene edited therapy for hemophilia A

- **Wilson's disease program**
  Indication: Wilson's Disease
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: ATP7B
  Gene editing therapy for Wilson's disease

MiMedx Group, Inc. (1 drugs)

Preclinical

- **EPIFIX**
  Indication: Advanced Wound Care
  Therapeutic Area: Wound Care
  Modality: Human-derived Allograft
  A human-derived allograft for external use in Advanced Wound Care applications

Moderna, Inc. (18 drugs)

Preclinical

- **mRNA-3139**
  Indication: Ornithine Transcarbamylase Deficiency
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy

Phase 1

- **mRNA-1405**
  Indication: Norovirus
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  mRNA-1405 is a pentavalent vaccine candidate against norovirus.

- **mRNA-1574**
  Indication: Human Immunodeficiency Virus (HIV)
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  mRNA-1574 is a vaccine candidate against HIV.

- **mRNA-1644**
  Indication: Human Immunodeficiency Virus (HIV)
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  mRNA-1644 is a vaccine candidate against HIV.

Phase 1/2

- **mRNA-3745**
  Indication: Glycogen storage disease type 1a (gsd1a)
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: glucose 6-phosphatase (G6Pase) enzyme
  mRNA-3745 consists of an mRNA encoding for modified human G6Pase and is designed to treat GSD1a.

- **mRNA-1018**
  Indication: Pandemic influenza
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  Pandemic influenza vaccine candidate

- **mRNA-4359**
  Indication: Melanoma, NSCLC
  Therapeutic Area: Oncology
  Modality: Oncology Therapeutic
  Target: Indoleamine 2,3-dioxygenase (IDO) And Programmed Death-ligand 1 (PD-L1) Antigens
  Checkpoint adaptive immune modulation therapy (AIM-T) candidate

- **mRNA-3705**
  Indication: Methylmalonic Acidemia (MMA)
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: Methylmalonyl-coa Mutase (MUT) Enzyme
  mRNA-3705 encodes for a missing or deficient hepatic enzyme and is designed to treat MMA.

- **mRNA-1769**
  Indication: Monkeypox
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  mRNA-1769 expresses four antigens from the monkeypox virus and has been shown to provide protection against multiple routes of infection with monkeypox virus.

- **mRNA-3927**
  Indication: Propionic acidemia (pa)
  Therapeutic Area: Rare Diseases
  Modality: Rare Disease Therapeutic
  Target: PCCA and PCCB subunit proteins
  PA therapeutic candidate

- **mRNA-1608**
  Indication: Herpes Simplex Virus Type 2 (HSV-2)
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  mRNA-1608 is a vaccine candidate against recurrent HSV-2 disease.

- **mRNA-1468**
  Indication: Varicella Zoster Virus (VZV)
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  mRNA-1468 is a vaccine candidate against shingles.

Phase 3

- **mRNA-1403**
  Indication: Norovirus Infection
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  Norovirus vaccine candidate

- **mRNA-4157**
  Indication: Melanoma, Non-Small Cell Lung Cancer (NSCLC), Bladder Cancer, Renal Cell Carcinoma
  Therapeutic Area: Oncology
  Modality: Oncology Therapeutic
  Target: Neoantigens
  Oncology therapeutic candidate

- **mRNA-1647**
  Indication: Cytomegalovirus (CMV) Infection
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  CMV vaccine candidate

- **mRNA-1010**
  Indication: Seasonal influenza
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  Seasonal influenza vaccine candidate

Approved

- **mRNA-1273**
  Indication: COVID-19
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  Original COVID vaccine

- **mRNA-1283**
  Indication: Covid-19
  Therapeutic Area: Infectious Diseases
  Modality: Vaccine
  Next-generation COVID-19 vaccine

Neurogene Inc. (2 drugs)

Phase 1/2

- **NGN-101**
  Indication: Cln5 Batten Disease
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Target: Cln5
  NGN-101 is a gene therapy product candidate for the treatment of CLN5 Batten disease, a rare neurological disorder, carrying the gene encoding human ceroid-lipofuscinosis neuronal protein 5 (CLN5).

Ocugen, Inc. (3 drugs)

Phase 2

- **OCU410**
  Indication: Geographic Atrophy (GA) Secondary To Dry Age-related Macular Degeneration (Damd)
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Target: RORA Gene
  A modifier gene therapy candidate being developed for the treatment of GA secondary to dAMD, utilizing a first-in-class approach by delivering the human RORA gene to diseased retinal tissue via subret...

Phase 2/3

- **OCU410ST**
  Indication: Stargardt disease
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Target: RORA gene
  A modifier gene therapy candidate being developed for the treatment of Stargardt disease, utilizing a first-in-class approach by delivering the human RORA gene to diseased retinal tissue via subretina...

Omeros Corporation (2 drugs)

Phase 2/3

- **zaltenibart**
  Indication: Complement 3 Glomerulopathy (C3g) And Other Alternative Pathway Disorders
  Therapeutic Area: Nephrology
  Modality: Monoclonal Antibody
  Target: MASP-3
  A monoclonal antibody targeting MASP-3 for the treatment of C3G and other alternative pathway disorders

- **zaltenibart (OMS906)**
  Indication: Paroxysmal Nocturnal Hemoglobinuria (PNH) And Complement 3 Glomerulopathy (C3G)
  Therapeutic Area: Immunology
  Modality: Monoclonal Antibody
  Target: MASP-3
  A proprietary, patented monoclonal antibody targeting MASP-3, the key activator of the alternative pathway of complement.

Opus Genetics, Inc. (2 drugs)

Phase 1/2

- **OPGx-LCA5**
  Indication: Leber congenital amaurosis (LCA) due to genetic variations in the LCA5 gene
  Therapeutic Area: Ophthalmology
  Modality: Gene therapy
  Target: LCA5 gene
  Gene therapy designed to address mutations in the LCA5 gene, which encodes the lebercilin protein, for the treatment of LCA5-associated inherited retinal disease (IRD), an early-onset retinal degenera...

- **OPGx-BEST1**
  Indication: Inherited Retinal Diseases (IRDs) Associated With Mutations In The BEST1 Gene (Best Disease)
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Target: BEST1 Gene
  Emerging gene therapy administered through a one-time subretinal injection

PTC Therapeutics, Inc. (3 drugs)

Phase 3

- **Translarna (ataluren)**
  Indication: Nonsense Mutation Duchenne Muscular Dystrophy (Nmdmd)
  Therapeutic Area: Neurology
  Modality: Small Molecule
  Translarna is an investigational new drug for the treatment of nmDMD.

Approved

- **Upstaza (eladocagene exuparvovec) / Kebilidi (eladocagene exuparvovec-tneq)**
  Indication: Aromatic l-amino decarboxylase (aadc) deficiency
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Upstaza/Kebilidi is a gene therapy for the treatment of AADC deficiency, a rare central nervous system disorder.

- **Upstaza (eladocagene exuparvovec)**
  Indication: Aromatic L-amino Decarboxylase (Aadc) Deficiency
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Upstaza is a gene therapy for the treatment of AADC deficiency.

Pacira BioSciences, Inc. (1 drugs)

Phase 2

- **PCRX-201**
  Indication: Osteoarthritis Of The Knee
  Therapeutic Area: Hematology
  Modality: Gene Therapy
  Target: IL-1R1
  A novel, locally administered gene therapy for the treatment of osteoarthritis of the knee

Passage Bio, Inc. (1 drugs)

Phio Pharmaceuticals Corp. (2 drugs)

Preclinical

- **PH-894**
  Therapeutic Area: Oncology
  Modality: Sirna
  Target: BRD4
  PH-894 is an INTASYL compound that is designed to silence BRD4, a protein that controls gene expression in both T cells and tumor cells.

Phase 1

- **PH-762**
  Indication: Cutaneous Squamous Cell Carcinoma, Melanoma And Merkel Cell Carcinoma
  Therapeutic Area: Oncology
  Modality: SiRNA
  Target: PD-1
  PH-762 is a potent RNAi molecule targeting PD-1. PH-762 can inhibit the immune checkpoint PD-1 in the tumor and thereby impede tumor growth.

Precigen, Inc. (2 drugs)

Phase 2

- **PRGN-2009**
  Indication: Tarp-associated Cancers
  Therapeutic Area: Oncology
  Modality: Gene Therapy
  Target: Hpv6 And Hpv11
  An investigational AdenoVerse immuno-therapy designed to activate the immune system to recognize and target human papillomavirus-positive (HPV+) solid tumors.

Phase 3

- **PRGN-2012**
  Indication: Recurrent Respiratory Papillomatosis (RRP)
  Therapeutic Area: Infectious Diseases
  Modality: Gene Therapy
  Target: HPV6 And HPV11
  An investigational AdenoVerse immunotherapy with optimized antigen design that uses gorilla adenovector technology to elicit immune responses directed against cells infected with HPV6 and HPV11.

Precision BioSciences, Inc. (5 drugs)

Preclinical

- **PBGENE-CNS**
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  In vivo gene editing program designed to achieve gene editing in the central nervous system

- **PBGENE-3243**
  Indication: Mitochondrial Disease
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: M.3243G Mitochondrial DNA (MtDNA)
  A potential treatment for m.3243 associated mitochondrial disease designed to specifically target and eliminate mutant m.3243G mitochondrial DNA (mtDNA).

- **PBGENE-DMD**
  Indication: Duchenne Muscular Dystrophy (Duchenne)
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: Dystrophin Gene
  Designed to improve function for more than 60% of patients afflicted with DMD by employing two complementary ARCUS nucleases delivered in a single AAV to excise exons 45-55 of the dystrophin gene.

- **PBGENE-LIVER**
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  In vivo gene editing program designed to achieve high efficiency gene insertion in nondividing cells

Phase 1

- **PBGENE-HBV**
  Indication: Chronic Hepatitis B (Chbv) Infection
  Therapeutic Area: Infectious Diseases
  Modality: Gene Therapy
  Target: Cccdna
  First-in-class in vivo gene editing therapy

Prime Medicine, Inc. (3 drugs)

Preclinical

- **Prime Editing-based treatment for Wilson's Disease**
  Indication: Wilson's disease
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  A Prime Editing-based treatment for Wilson's Disease, currently in preclinical development.

- **Prime Editing-based treatment for AATD**
  Indication: Alpha-1 Antitrypsin Deficiency
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  A Prime Editing-based treatment for AATD, currently in preclinical development.

Phase 1/2

- **PM359**
  Indication: Chronic Granulomatous Disease (CGD)
  Therapeutic Area: Immunology
  Modality: Gene Therapy
  A Prime Editing-based treatment for CGD, which led to 58% dihydrorhodamine positivity by Day 15, 66% by Day 30 and 71% by Day 60 in the first patient dosed. This is an open-label, single-arm, multicen...

Pyxis Oncology, Inc. (1 drugs)

Preclinical

- **PYX-203**
  Indication: Hematologic Cancers
  Therapeutic Area: Oncology
  Modality: Antibody-Drug Conjugate (ADC)
  Target: CD123
  An investigational ADC that targets and binds to the interleukin-3 receptor, also known as CD123, a rapidly internalizing target that is overexpressed in hematologic cancers.

Q32 Bio Inc. (1 drugs)

Phase 1

- **ADX-097**
  Indication: Complement Mediated Diseases
  Therapeutic Area: Immunology
  Modality: Monoclonal Antibody
  Target: C3
  An anti-C3d antibody linked to two moieties of a fragment of human factor H, designed to inhibit alternative pathway complement activation locally in diseased tissues.

REGENXBIO Inc. (4 drugs)

Phase 1/2

- **RGX-111**
  Indication: Mucopolysaccharidosis Type I (MPS I), Also Known As Hurler Syndrome
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: IDUA gene
  RGX-111 is a gene therapy designed to deliver a functional copy of the alpha-L-iduronidase gene (IDUA) to the central nervous system, being developed for the treatment of MPS I. However, its developme...

Phase 2/3

- **RGX-202**
  Indication: Duchenne Muscular Dystrophy (Duchenne)
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  RGX-202 is a gene therapy designed to deliver a transgene for a novel microdystrophin, being developed for the treatment of Duchenne.

- **ABBV-RGX-314**
  Indication: Wet Age-Related Macular Degeneration (Wet Amd)
  Therapeutic Area: Ophthalmology
  Modality: Gene Therapy
  Target: Vascular Endothelial Growth Factor-C (VEGF-C)
  ABBV-RGX-314 is a gene therapy designed to deliver a therapeutic antibody fragment to inhibit VEGF, being developed for the treatment of wet AMD and DR.

- **RGX-121**
  Indication: Mucopolysaccharidosis Type I (MPS I), Also Known As Hurler Syndrome
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: Iduronate-2-sulfatase Enzyme
  Clemidsogene lanparvovec (RGX-121) on track to be first gene therapy and one-time treatment for MPS II

Recursion Pharmaceuticals, Inc. (1 drugs)

Phase 1/2

- **REC-1245**
  Indication: Advanced Solid Tumors
  Therapeutic Area: Oncology
  Modality: Small Molecule
  Target: RBM39
  Potential first-in-class oral RBM39 degrader that selectively impairs alternative splicing to silence multiple DDR pathways, leading to high replication stress.

Rocket Pharmaceuticals, Inc. (6 drugs)

Phase 1

- **RP-L301**
  Indication: Pyruvate Kinase Deficiency
  Therapeutic Area: Dermatology
  Modality: Gene Therapy
  Target: PKLR
  RP-L301 is an investigational gene therapy for the treatment of Pyruvate Kinase Deficiency, containing autologous hematopoietic stem cells that have been genetically modified with a lentiviral vector ...

- **RP-L102**

  Indication: Fanconi Anemia (FA)

  Therapeutic Area: Dermatology

  Modality: Gene Therapy

  Ex vivo lentiviral-based gene therapy for the treatment of FA

- **RP-A701**

  Indication: Bag3 Dilated Cardiomyopathy

  Therapeutic Area: Cardiovascular

  Modality: Gene Therapy

  Target: Bag3

  RP-A701 is an AAV.rh74-based gene therapy candidate for the treatment of BAG3 Dilated Cardiomyopathy, designed to deliver a fully functional BAG3 gene to augment BAG3 protein levels in cardiomyocytes.

- **RP-A601**

  Indication: Plakophilin-2 Arrhythmogenic Cardiomyopathy

  Therapeutic Area: Cardiovascular

  Modality: Gene Therapy

  Target: Pkp2

  RP-A601 is an investigational gene therapy for the treatment of Plakophilin-2 Arrhythmogenic Cardiomyopathy, consisting of a recombinant adeno-associated serotype rh74 capsid containing a functional v...

Phase 2

- **RP-L201**

  Indication: Leukocyte adhesion deficiency-i (lad-i)

  Therapeutic Area: Immunology

  Modality: Gene Therapy

  Target: ITGB2

  RP-L201 is an investigational gene therapy for the treatment of Leukocyte Adhesion Deficiency-I, containing autologous hematopoietic stem cells that have been genetically modified with a lentiviral ve...

- **RP-A501**

  Indication: Danon Disease

  Therapeutic Area: Cardiovascular

  Modality: Gene Therapy

  Target: Lamp2

  RP-A501 is an investigational gene therapy for the treatment of Danon disease, consisting of a recombinant adeno-associated serotype 9 (AAV9) capsid containing a full-length, wild-type version of the ...

Sana Biotechnology, Inc. (1 drugs)

Preclinical

- **SG299**
  Indication: B-cell Cancers And B-cell Mediated Autoimmune Diseases
  Therapeutic Area: Oncology
  Modality: Gene Therapy
  Target: Cd19
  A CD8-targeted fusosome that delivers genetic material to make CD19-directed CAR T cells.

Sangamo Therapeutics, Inc. (4 drugs)

Preclinical

- **ST-506**
  Indication: prion disease
  Therapeutic Area: Neurology
  Modality: Gene therapy
  An investigational epigenetic regulator for the treatment of prion disease, leveraging STAC-BBB.

- **Prion disease product candidate**
  Indication: Prion Disease
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  An epigenetic regulation product candidate to treat prion disease, leveraging the novel proprietary neurotropic adeno-associated virus (AAV) capsid variant, STAC-BBB.

Phase 1

- **ST-503**
  Indication: Chronic Neuropathic Pain
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Target: Nav1.7
  An investigational epigenetic regulator for the treatment of intractable pain due to idiopathic small fiber neuropathy, a type of chronic neuropathic pain. It is intended to deliver a modified copy of...

Sarepta Therapeutics, Inc. (3 drugs)

Phase 3

- **SRP-9003**
  Indication: Lgmd2e (beta-sarcoglycanopathy)
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: Beta-sarcoglycan gene
  SRP-9003 is a gene therapy that uses an AAV vector to express the beta-sarcoglycan protein.

Approved

- **ELEVIDYS**
  Indication: Duchenne
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: Dystrophin Gene
  ELEVIDYS (delandistrogene moxeparvovec-rokl) is the only approved gene therapy for Duchenne muscular dystrophy (DMD) with a boxed warning for the risk of acute serious liver injury (ALI) and acute liv...

Senti Biosciences, Inc. (1 drugs)

Preclinical

- **SENTI-301A**
  Indication: Solid Tumors
  Therapeutic Area: Oncology
  Modality: Gene Therapy
  A gene circuit incorporated into Celest's CAR-NK cells

Silence Therapeutics plc (3 drugs)

Preclinical

- **SLN548**
  Therapeutic Area: Immunology
  Modality: Sirna
  SLN548 is a wholly owned siRNA for complement-mediated diseases, but its development has been paused.

Phase 2

- **Divesiran**
  Indication: Polycythemia Vera (PV)
  Therapeutic Area: Dermatology
  Modality: SiRNA
  Target: TMPRSS6
  Divesiran is a siRNA product candidate designed to inhibit TMPRSS6 expression in the liver, aiming to increase hepcidin production and reduce excessive red blood cell production in polycythemia vera p...

Phase 3

- **Zerlasiran**
  Indication: Cardiovascular disease
  Therapeutic Area: Cardiovascular
  Modality: Sirna
  Target: LPA
  Zerlasiran works by temporarily silencing LPA expression in the liver, lowering the body's production of apolipoprotein(a), a key component of lipoprotein(a).

Solid Biosciences Inc. (6 drugs)

Preclinical

- **SGT-701**
  Indication: RBM20 DCM
  Therapeutic Area: Cardiovascular
  Modality: Gene Therapy
  Target: RBM20

- **SGT-401**
  Indication: BAG3-Mediated DCM
  Therapeutic Area: Cardiovascular
  Modality: Gene Therapy
  Target: BAG3

- **SGT-601**
  Indication: TNNT2-Mediated Dilated Cardiomyopathy
  Therapeutic Area: Cardiovascular
  Modality: Gene Therapy
  Target: TNNT2
  SGT-601 is a gene therapy candidate for the treatment of TNNT2-mediated dilated cardiomyopathy.

Phase 1

- **SGT-212**
  Indication: Friedreich Ataxia
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Target: FXN
  SGT-212 is a gene therapy candidate for the treatment of Friedreich's ataxia.

- **SGT-501**
  Indication: Catecholaminergic Polymorphic Ventricular Tachycardia
  Therapeutic Area: Cardiovascular
  Modality: Gene Therapy
  Target: Casq2
  SGT-501 is a gene therapy candidate for the treatment of catecholaminergic polymorphic ventricular tachycardia.

Phase 3

- **SGT-003**
  Indication: Duchenne muscular dystrophy
  Therapeutic Area: Neurology
  Modality: Gene therapy
  SGT-003 is a gene therapy candidate for the treatment of Duchenne muscular dystrophy.

Stoke Therapeutics, Inc. (2 drugs)

Phase 3

- **zorevunersen (STK-001)**
  Indication: Dravet Syndrome
  Therapeutic Area: Neurology
  Modality: Antisense Oligonucleotide
  Target: SCN1A
  A potential disease-modifying medicine for the treatment of Dravet syndrome, a severe and progressive genetic epilepsy. Zorevunersen is an investigational new medicine for the treatment of Dravet synd...

- **zorevunersen**
  Indication: Dravet Syndrome
  Therapeutic Area: Neurology
  Modality: Antisense Oligonucleotide
  Target: SCN1A
  Zorevunersen is an antisense oligonucleotide being developed for the treatment of Dravet syndrome. Zorevunersen is an investigational new medicine for the treatment of Dravet syndrome. It is an antise...

Taysha Gene Therapies, Inc. (4 drugs)

Preclinical

- **TSHA-118**
  Indication: Cln1 disease
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Target: CLN1
  A self-complementary AAV9 viral vector that expresses human codon-optimized CLN1 complementary deoxyribonucleic acid, for the treatment of CLN1 disease, a progressive, fatal neurodegenerative disease.

- **TSHA-105**
  Indication: Slc13a5 Deficiency
  Therapeutic Area: Neurology
  Modality: Gene Therapy

- **TSHA-113**
  Indication: Tauopathies
  Therapeutic Area: Neurology
  Modality: Gene Therapy
  Target: Tau
  An AAV9 capsid that packages a tau-specific miRNA, delivered in the cerebrospinal fluid for the treatment of tauopathies.

Tenaya Therapeutics, Inc. (2 drugs)

Phase 1

- **TN-401**
  Indication: Pkp2-associated Arrhythmogenic Right Ventricular Cardiomyopathy (ARVC)
  Therapeutic Area: Cardiovascular
  Modality: Gene Therapy
  Target: Pkp2
  TN-401 is a gene therapy designed to deliver a working PKP2 gene to specific cells of the heart to produce plakophilin protein and potentially slow or reverse the course of PKP2-associated ARVC.

TransCode Therapeutics, Inc. (4 drugs)

Preclinical

- **TTX-RIGA**
  Therapeutic Area: Oncology
  Modality: Sirna
  Target: RIG-I
  TTX-RIGA is an RNA-based agonist of the retinoic acid-inducible gene I (RIG-I), targeting activation of innate immunity in the tumor microenvironment.

- **TTX-siMYC**
  Therapeutic Area: Oncology
  Modality: Sirna
  Target: c-MYC
  TTX-siMYC is an siRNA-based inhibitor of c-MYC, a widely expressed but currently undruggable oncogene.

- **TTX-siPDL1**
  Therapeutic Area: Oncology
  Modality: Sirna
  Target: PD-L1
  TTX-siPDL1 is an siRNA-based modulator of programmed death-ligand 1 (PD-L1).

Phase 1/2

- **TTX-MC138**
  Indication: Advanced Solid Tumors
  Therapeutic Area: Oncology
  Modality: Sirna
  Target: Mir-10b
  TTX-MC138 is an siRNA-based therapeutic candidate that targets microRNA-10b, a master regulator of metastatic cell viability in various cancers.

Ultragenyx Pharmaceutical Inc. (5 drugs)

Phase 1/2

- **UX701 (rivunatpagene miziparvovec)**
  Indication: Wilson's Disease
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: ATP7B
  UX701 is an AAV type 9 gene therapy, administered by a one-time IV infusion that is designed to deliver a truncated form of the ATP7B gene.

Phase 3

- **DTX401 (pariglasgene brecaparvovec)**
  Indication: Glycogen Storage Disease Type Ia
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: G6Pase-
  DTX401 is an adeno-associated virus 8, or AAV8, gene therapy clinical candidate, administered by a one-time IV infusion that is designed to deliver stable expression and activity of G6Pase-.

- **DTX301 (avalotcagene ontaparvovec)**
  Indication: Ornithine Transcarbamylase Deficiency
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: OTC
  DTX301 is an AAV8 gene therapy product candidate, administered by a one-time IV infusion that is designed to deliver stable expression and activity of the OTC.

- **GTX-102**
  Indication: Angelman Syndrome
  Therapeutic Area: Neurology
  Modality: Antisense Oligonucleotide
  Target: Paternal UBE3A Antisense
  GTX-102 is an antisense oligonucleotide, or ASO, administered by intrathecal injection that inhibits expression of the paternal UBE3A antisense.

Filed

- **UX111 (rebisufligene etisparvovec)**
  Indication: Sanfilippo Syndrome Type A
  Therapeutic Area: Rare Diseases
  Modality: Gene Therapy
  Target: Heparan Sulfate
  UX111 is an adeno-associated virus 9, or AAV9, gene therapy product candidate, administered by a one-time IV infusion that provides the cross-correcting enzyme that enables the breakdown of Heparan su...

Veracyte, Inc. (5 drugs)

Preclinical

- **Percepta Nasal Swab test**
  Indication: Lung Cancer
  Therapeutic Area: Oncology
  Modality: Gene Therapy
  A noninvasive test that helps physicians more accurately determine lung cancer risk in patients with lung nodules found on CT scans.

- **Decipher Prostate Genomic Classifier**
  Indication: Prostate Cancer
  Therapeutic Area: Oncology
  Modality: Gene Therapy
  A genomic test that helps physicians personalize therapy for patients with prostate cancer, predicting the risk of progressing to metastatic disease within five years.

- **Envisia Genomic Classifier**
  Indication: Interstitial Lung Disease
  Therapeutic Area: Respiratory
  Modality: Gene Therapy
  A genomic test for improving the diagnosis of interstitial lung diseases without the need for surgery.

- **Afirma Genomic Sequencing Classifier**
  Indication: Thyroid Cancer
  Therapeutic Area: Oncology
  Modality: Gene Therapy
  A genomic test that determines which patients with indeterminate thyroid nodule biopsy results are actually benign, helping them avoid unnecessary surgery.

- **Decipher Bladder Genomic Classifier**
  Indication: Bladder cancer
  Therapeutic Area: Oncology
  Modality: Gene Therapy
  A genomic test that measures the molecular profile of bladder cancer, helping physicians determine which patients are most likely to benefit from neoadjuvant chemotherapy.

Vertex Pharmaceuticals Incorporated (3 drugs)

Phase 1/2

- **VX-522**
  Indication: Cystic Fibrosis
  Therapeutic Area: Respiratory
  Modality: MRNA Therapy
  Target: CFTR
  A nebulized CFTR mRNA therapy for the treatment of people with CF who do not produce full-length CFTR protein.

Approved

- **CASGEVY**
  Indication: Sickle Cell Disease (SCD) And Transfusion-dependent Beta Thalassemia (TDT)
  Therapeutic Area: Dermatology
  Modality: Gene Therapy
  Target: HBB
  An ex-vivo, non-viral CRISPR/Cas9 gene-edited cell therapy for the treatment of people 12 years of age and older with SCD or TDT.

- **JOURNAVX**
  Indication: Acute Pain
  Therapeutic Area: Pain
  Modality: Small Molecule
  Target: Nav1.8
  A selective non-opioid NaV1.8 pain signal inhibitor for the treatment of people with moderate-to-severe acute pain.

Voyager Therapeutics, Inc. (1 drugs)

bioAffinity Technologies, Inc. (1 drugs)

Preclinical

- **siRNA product candidates**
  Indication: Cancer
  Therapeutic Area: Oncology
  Modality: SiRNA
  Target: CD320 And LRP2
  siRNA product candidates for the treatment of cancer, targeting CD320 and LRP2 cell membrane proteins

enGene Holdings Inc. (1 drugs)

Disclaimer

Information and Educational Purposes Only

This report is provided for informational and educational purposes only and does not constitute medical, investment, financial, or professional advice of any kind. The information contained herein should not be used as a basis for making investment decisions, medical treatment decisions, or any other decisions that could affect your health or financial well-being. PharmExplore makes no representations or warranties regarding the accuracy, completeness, or reliability of the information presented. Readers are strongly advised to consult with qualified healthcare professionals, financial advisors, or other appropriate experts before making any decisions based on the content of this report.

The data presented in this report has been aggregated and processed from publicly available sources using artificial intelligence and automated data scraping technologies. Sources include, but are not limited to, ClinicalTrials.gov, company press releases, SEC filings, patent databases, and other third-party sources. The accuracy, completeness, and timeliness of this data may vary significantly due to the automated nature of data collection and processing. Information may be outdated, incomplete, misinterpreted, or subject to change without notice. This landscape analysis does not capture private companies, confidential development programs, or non-publicly disclosed pipeline assets, and therefore does not represent a comprehensive view of the therapeutic landscape. Drug development timelines, regulatory statuses, and clinical outcomes are subject to change and inherently uncertain. Past performance and current development status are not indicative of future results. PharmExplore disclaims all liability for any actions taken or not taken based on the information contained in this report.

Report generated on November 26, 2025