Timing of FT Designation: Recent Products, CBER (Gene/Cell Therapies, Vaccines)

Product	Indication	Grant Date	Stage
Quince's EryDex System (dexamethasone sodium phosphate encapsulated in a patient's own red blood cells and then re-infused)	Ataxia-telaniectasia (designed to provide efficacy of corticosteroids but to reduce/ eliminate significant AEs that accompany chronic corticosteroid therapy)	6/3/2024	P3 pivotal initiated. Data from prior P3 showed encouraging efficacy.
Theriva's VCN-01 (oncolytic virus) in comb w/ gemcitabine and nab-paclitaxel	Metastatic pancreatic adenocarcinoma	5/23/2024	In P2b (initiated 4Q2022). In Feb '22, independent data monitoring committee recommended continuation of P2b study providing first evidence of possibility of repeat dosing
iECURE's ECUR-506 (gene editing therapy)	Neonatal onset ornithine Transcarbamylase (OTC) deficiency	5/7/2024	Based on preclinical results. FIH P1/2 study now actively enrolling
Lexeo's LX2006 (AAV-based gene therapy)	Friederich's ataxia cardiomyopathy	4/16/2024	Based on "available preclinical data"
Indapta's IDP-023 (allogeneic G-NK cell therapy)	NHL and Myeloma	2/29/2024	In P1. Initial P1 data expected 2H2024
Cabaletta's CABA-201 (CAR T cell therapy) (two FT designations)	Dermatomyositis to improve disease activity and for systemic sclerosis to improve associated organ dysfunction	1/8/2024	In 4 P1/2 trials (9 cohorts), including for dermatomyositis. In preclinical studies for skin/ organ cohort, with cleared IND
MimiVax's SurVaxM	Newly diagnosed glioblastoma	10/13/2023	Based on results from P2a SURVIVE trial
Artiva's AlloNK (AB-101) in comb w/ rituximab or obinotuzumab (OTS allogeneic natural killer cell-based therapy)	Lupus nephritis	2/22/2024	Currently in P1, open-label study in LN patients unresponsive to/relapsed following standard therapy
Candel's CAN-3110 (HSV-1 oncolytic viral immunotherapy)	Recurrent high-grade glioma	2/13/2024	In investigator-sponsored P1b study: In published study, found nearly a doubling of expected median OS after single injection, achieving a mOS of ~12 months, compared to historical reports of less than 6-9 months in this therapy-resistant condition. Survival in CAN-3110 group >14 months.
Krystal's Inhaled KB707 (modified HSV-1 vector delivering genes encoding both human IL-12 and IL-2 to tumor microenvironment)	Solid tumors w/ pulmonary metastases R/R to SOC therapy	2/13/2024	Based on PC data in stringent syngeneic mouse models
Ultimovacs' UV1 in comb w/ ipilimumab/ nivolumab (cancer vaccine)	Treatment of patients w/ unresectable malignant pleural mesothelioma	2/5/2024	Based on results of randomized P2 trial showing improved OS when vax used as add-on to IPI or Nivo immunotherapy
Biosyngen's BST02 (TIL cell therapy)	Locally advanced liver cancers, including hepatocellular carcinoma and cholangiocarcinoma, to improve overall survival	2/1/2024	Appears based on preclinical data. FDA cleared IND for P1/2 trial in October 2023
Kyverna's KYV-101 (autologous CAR T-cell therapy)	Refractory progressive MS	1/19/2024	In P1. On 1/4/2024, company announced FDA IND clearance for P2 study in patients w/ refractory, progressive MS
Lexeo's LX2020 (AAV-based gene therapy)	PKP2 arrhythmogenic cardiomyopathy (ACM)	12/18/2023	Appears based on preclinical data. Fast track announcement discussed "planned P1/2 trial."
Kyverna's KYV-101 (autologous CAR T-cell therapy)	Refractory myasthenia gravis	12/13/2023	Appears to be supported by P1 KYSA-1 (US) and P1/2 KYSA-3 (Germany) studies in lupus nephritis. In November 2023, Kyvera rec'd FDA IND clearance for KYV-101 to be used in P2 open-label study in myasthenia gravis. Appears supported by 20-patient P1/2 oncology study that highlighted KY-101's potential in autoimmune diseases.
Solid's SGT-003 (gene therapy using rationally designed capsid to deliver DNA sequence encoding for a shortened form of dystrophin protein)	Duchenne muscular dystrophy	12/7/2023	Appears based upon preclinical data. FDA IND clearance was rec'd in Nov 2023 for planned P1/2 FIH trial for 1x IV infusion in 2 cohorts w/ minimum of 3 patients each
CG Oncology's CG0070 (oncolytic immunotherapy) (Fast Track designation announced with Breakthrough designation)	High-risk Bacillus Calmette-Guerin (cancer) unresponsive non-muscle invasive bladder cancer with carcinoma in situ with/without Ta or T1 (papillary) tumors	12/5/2023	Appears based on "interim analysis" (presented 11/30/23) of P3 BOND-003 single-arm study in patients evaluable for efficacy w/ minimum 3-month follow-up (n=66): • Treated patients had complete response (CR) of 75.7% at any time (50/66). • The 3- and 6-month landmark CR rates were 68.2% (45/66) and 63.6% (42/66), respectively.
Aspen's ANPD001 (personalized autologous cell therapy)	Parkinson's disease to improve motor function (by replacing lost dopamine neurons)	10/19/2023	Appears based on preclinical IND-enabling studies. At time of FT announcement, company detailed plans for 1st in patients (moderate to severe PD) P1/2a trial following the 2022 Trial-Ready Screening Cohort Study to screen, enroll, and begin manufacturing cells for the clinical trial.
Diakonos Oncology's DOC1021 (dendritic cell vaccine)	Glioblastoma multiforme	10/15/2023	Based on "positive preliminary" safety and efficacy data from a P1 study
Biosyngen's BRG01 (adoptive immune cell therapy)	Epstein-Barr virus-positive R/R nasopharyngeal carcinoma	07/26/2023	In P1
Krystal's KB707 (intratumoral) (modified HSV-1 vector delivering genes encoding both human IL-12 and IL-2 to tumor microenvironment)	To delay disease progression in treating patients w/ anti-PD-1 R/R locally advanced or metastatic melanoma	7/17/2023	Appears based on preclinical data (company announced at time that it expects to dose 1st patient in 2H2023)
Genprex's Reqorsa (gene therapy) in comb w/ Tecentriq	Extensive-stage SCLC	07/11/2023	Expects to begin P1/2 enrollment 3Q2023
Kyverna's KYV-101 (CAR T-cell therapy)	Refractory lupus nephritis	07/01/2023	Actively enrolling P1 trial (US) and has filed CTA for German P1/2 trial in lupus nephritis. Also appears supported by 20-patient P1/2 oncology study that highlighted KY-101's potential in autoimmune diseases.
GSK's Neisseria-gonorrhoea vaccine	Prevention of gonorrhoea	06/07/2023	Appears based on P1 healthy volunteer dose-escalation safety lead-in study. P2 POC study ongoing.
Tenaya's TN-201 (AAV-based gene therapy)	Treatment of myosin-binding protein C3-assoc (mutations) hypertrophic cardiomyopathy	05/02/2023	Appears based on preclinical studies. IND cleared January 2023.
Cabaletta's CABA-201 (CAR-T cell therapy)	Systemic lupus erythematosus	05/1/2023	Initiating P1/2
RegenexBio's RGX-202 (gene therapy)	Duchenne muscular dystrophy	04/11/2023	Recruiting for P1/2 study
Caribou's CB-011 (allogeneic CAR-T cell therapy)	R/R MM	04/04/2023	Appears based on preclinical data as 1st patient dosed around time of designation
Artiva's AB-101 (allogeneic NK cell-based therapy) in comb w/ rituximab	R/R NHL of B-cell origin	1/31/2023	In P1/2