Recent Advanced Therapy BTDs w/ Basis for Designations

Product	Indication	Date of BTD	Response Rates Used to Support BTD
Seres' SER-155 (microbiome therapy—consortium of bacterial strains)	To reduce risk of infection and GvHD in allo-HSCT patients	1/9/2024	Based on preclinical and P1b Cohort 1 data showing favorable tolerability, successful drug bacteria engraftment, and a substantial reduction in pathogen domination in GI microbiome vs. a reference cohort of patients
CG Oncology's CG0070 (oncolytic immunotherapy) (BTD announced w/ Fast Track 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 "nterim 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) rate 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.
Inovio's INO-3107 (DNA medicine comprising plasmids encoding for HPV-6, HPV-11, and human interleukin-12 engineered w/ Inovio's SynCon technology and delivered using the Cellectra 5PSP intradermal electroporation device)	Recurrent respiratory papillomatosis (RRP)	9/7/2023	Supported by data from completed P1/2 open-label trial in patients w/ HPV-6 and/or HPV-11-related RRP: • Overall, 81.3% (26/32) of patients had declines in surgical interventions in year post-administration vs. prior year, including 28.1% (9/32) who req'd no surgical intervention during/after dosing window. • Post-dosing, there was median decrease of 3 surgical interventions (95% CI) (patients had median range of 4 surgeries (range: 2-8) in year prior to dosing)
Precigen's PRGN-2012 AdenoVerse (therapeutic vaccine)	Recurrent respiratory papillomatosis (RRP)	06/20/2023	In P1 patients (n=15) at recommended dose Level 2 (n=12), who had average of 5.8 RRP surgeries (range: 3-10) in prior year: • 50% (6/12) had complete response (no post-treatment surgeries needed w/minimum follow-up of 12 months) • Reduction of surgeries in 83% of patients (10/12) over 12 months following treatment.
Merck/Moderna's mRNA-4157/V940 in comb w/ Keytruda (personalized cancer vaccine)	Adjuvant treatment of high-risk melanoma following complete resection	2/22/2023	Based on ongoing open-label P2b study in the overall ITT population of 157 patients: • Combo therapy reduced the risk of recurrence or death by 44% compared to Keytruda alone. Recurrence or death was reported in 22.4% of patients in combo arm (n=24/107) compared with 40% (n=20/50) receiving Keytruda alone. • The 12-month RFS rate was 83.4% and 77.1% in the combo and control arms, respectively. The 18-month RFS rate was 78.6% and 62.2% in the combo and control arms, respectively.
Moderna's mRNA-1345 (vaccine)	Prevention of RSV-associated lower respiratory tract disease (RSV-LRTD) in adults 60 and older	1/30/2023	Based on "positive topline data" from randomized/DB/PC ConqerRSV P3 pivotal study evaluating efficacy against RSV-LRTD as defined by 2 or more symptoms. Study met its primary efficacy endpoints, including vaccine efficacy of 83.7% (95.88% CI) against RSV-LRTD and vaccine efficacy of 82.4% (96.36% CI) in RSV-LRTD as defined by three or more symptoms.
Vaxcyte's V-24 (vaccine)	24-valent pneumococcal conjugate vaccine for prevention of invasive pneumococcal disease	1/5/2023	Based on "positive topline results" from P1/2 POC study evaluating safety, efficacy, tolerability, and immunogenicity in adults 18-64: • VAX-24 met/exceeded the established regulatory immunogenicity standards for all 24 serotypes at 2.2 mcg dose (w/ safety profile similar to Prevnar 20), which firm plans to use in pivotal P3. • At this dose, VAX-24 met the standard opsonophagocytic activity response non-inferiority criteria for all 20 serotypes common w/ Prevnar 20, of which 16 achieved higher immune responses.