Demographic Data Summary for Novel Biological Approvals

January 1, 2022 – December 31, 2022

Overview

FDA's Center for Biologics Evaluation and Research (CBER) regulates complex biological products, such as preventive vaccines for infectious diseases, blood products, and cell and gene therapy products, the majority of which are indicated for rare diseases. In 2022 CBER approved eight novel biological products, including two vaccines, one live biotherapeutic product and five cell and gene therapy products. Five of these eight biological approvals had orphan-drug designation.

This inaugural Demographic Data Summary highlights certain demographic information about the participants of the key clinical trial(s) that supported approval of each of these eight novel biological products. Specifically, the summary provides the percentages of clinical trial participants by sex (female), race (White, Black, and Asian), ethnicity (Hispanic) and age (≥ 65 years of age).

The demographic information provided for each approval in this summary can be found in publicly available FDA reviews and other approval documents on FDA's website. Information about other clinical trials that may have been conducted in different study populations for a listed biological approval may be available in the approval materials posted by FDA.

The demographic information provided in this summary is organized in tables by the biological product type categories listed below with footnotes that apply to all three tables at the end.

- **Vaccines**
- **Live Biotherapeutics**
- **Cell and Gene Therapies**

Table 1. Vaccines

Trade Name	Non-proprietary Name	Indication	Total N	% Female	% White†	% Black†	% Asian†	% Hispanic‡	% ≥ 65 years unless otherwise denoted
PRIORIX[S, PI]	Measles, Mumps and Rubella Vaccine, Live	Active immunization for the prevention of measles, mumps, and rubella in individuals 12 months of age and older	12,151	48.4	64.6	6.1	18.4	14.3	0
SPIKEVAX[E, PI]	COVID-19 Vaccine, mRNA	Active immunization against coronavirus disease 2019 (COVID-19) caused by the SARS-CoV-2 virus in persons 18 years of age and older	28, 451	47.5	79.7	9.7	4.7	19.7	25.4

Table 2. Live Biotherapeutics

Trade Name	Non-proprietary Name	Indication	Total N	% Female	% White†	% Black†	% Asian†	% Hispanic‡	% ≥ 65 years unless otherwise denoted
REBYOTA*[, S, PI]	Fecal microbiota, live-jslm	for the prevention of recurrence of *Clostridioides difficile* infection (CDI) in individuals 18 years of age and older, following antibiotic treatment for recurrent CDI	978	67.2	93.8	NA	NA	2.4	48.8

Table 3. Cell and Gene Therapy Products

Trade Name	Non-proprietary Name	Indication	Total N	% Female	% White†	% Black†	% Asian†	% Hispanic‡	% ≥ 65 years unless otherwise denoted
CARVYKTI*, E, S, CL	ciltacabtagene autoleucel	Treatment of adult patients with relapsed or refractory multiple myeloma, who previously received a proteasome inhibitor (PI), an immunomodulatory agent (IMiD) and an anti-CD38 antibody	97	41.2	71	18	1	6.2	36
ZYNTEGLO*E, CL	betibeglogene autotemcel	For treatment of adult and pediatric patients with ß-thalassemia who require regular red blood cell (RBC) transfusions	41	49	44	NA	49	2	0
SKYSONA*S, PI	elivaldogene autotemcel	To slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD)	67	0	54	4	1	25	0
HEMGENIX*E, CL, ST	etranacogene dezaparvovec-drlb	For treatment of adults with Hemophilia B (congenital Factor IX deficiency) who: •Currently use Factor IX	54	0	74.1	1.9	3.7	7.4	14.8 (≥ 60 years)

		prophylaxis therapy, or •Have current or historical life-threatening hemorrhage, or •Have repeated, serious spontaneous bleeding episodes							
ADSTILADRIN[E, CL]	nadofaragene firadenovec-vncg	For the treatment of adult patients with high-risk Bacillus Calmette-Guérin (BCG)-unresponsive non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) with or without papillary tumors	103	11.7%	92.2%	5.8%	1.9%	2.9%	76.7%

Footnotes (apply to all tables)**:**
NA – not available
*Orphan-designated drug
† The percentages of all other races combined (American Indian, Alaska Native, Native Hawaiian or other Pacific Islander, Other, Unknown/Unreported) adds up to 100% of race category.
‡ The percentage of Non-Hispanic and Unknown/Unreported ethnicity adds up to 100% of ethnicity category.
[S] Demographic data are from the population analyzed for safety.
[E] Demographic data are from the population analyzed for efficacy.
[PI] The demographic information provided here can be found in the Package Insert (PI).
[CL] The demographic information provided here can be found in the Clinical Review Memo.
[ST] The demographic information provided here can be found in the Statistical Review Memo.