

Full references' list

	Product	Indication	HTA	HTA reference
1	Strimvelis	ADA-SCID	NICE	National Institute for Health and Care Excellence. Strimvelis for treating adenosine deaminase deficiency–severe combined immunodeficiency: Highly specialised technology guidance. London: National Institute for Health and Care Excellence (NICE); 2018 Feb 07.
2	Zynteglo	Beta-thalassemia	NICE	National Institute for Health and Care Excellence. Betibeglogene autotemcel for treating transfusion-dependent beta-thalassaemia: Appraisal consultation document. London: National Institute for Health and Care Excellence (NICE); 2021 Feb.
3	Zynteglo	Beta-thalassemia	ICER	Institute for Clinical and Economic Review. Betibeglogene Autotemcel for Beta Thalassemia: Effectiveness and Value: Final evidence report. Boston: ICER; 2022 Jul 19.
4	Zynteglo	Beta-thalassemia	HAS	Avis d'efficience. Zynteglo Bluebird bio). Paris: Haute Autorité de Santé (HAS). 2020 Feb 11.
5	Zynteglo	Beta-thalassemia	G-BA	Benefit assessment of medicinal products with new active ingredients according to section 35a SGB V: Betibeglogene Autotemcel. Gemeinsamer bundesausschuss (G-BA). 2020 Dec 3.
6	Libmeldy	MLD	NICE	National Institute for Health and Care Excellence. Atidarsagene autotemcel for treating metachromatic leukodystrophy: Highly specialised technology guidance. London: National Institute for Health and Care Excellence (NICE); 2023 Mar 28.
7	Libmeldy	MLD	ICER	Institute for Clinical and Economic Review. Atidarsagene Autotemcel for Metachromatic Leukodystrophy: Final Evidence report. Boston: ICER; 2023 Oct 30.
8	Libmeldy	MLD	HAS	Transparency Committee Summary. Autologous CD34+ cell enriched population that contains haematopoietic stem and progenitor cells (HSPC) transduced ex vivo using a lentiviral vector encoding the human arylsulfatase A (ARSA) gene LIBMELDY, 2-10 x 10 ⁶ cells/mL dispersion for infusion. Paris: Haute Autorité de Santé (HAS). 2021 Apr 21.
9	Libmeldy	MLD	G-BA	Bewertung von Arzneimitteln für seltene Leiden nach § 35a Absatz 1 Satz 11 i. V. m. Kapitel 5 § 12 Nr. 1 Satz 2 Verfo Wirkstoff: Atidarsagene autotemcel/OTL-200. 2021 Aug 2.
10	Libmeldy	MLD	AIFA	Scientific Report. Libmeldy (Atidarsagene autotemcel): Italian Medicines Agency (AIFA). 2022 May 20.
11	Libmeldy	MLD	AEMPS	Informe de Posicionamiento Terapéutico de Atidarsagene autotemcel (Libmeldy) en de la leucodistrofia metacromatica: AEMPS. 2023 Sep 01.

12	Casgevy	Beta-thalassemia	ICER	Institute for Clinical and Economic Review. Gene therapies for sickle cell disease: Evidence report. Boston: ICER; 2023 Jul 13.
13	Casgevy	Sickle cell disease	ICER	Institute for Clinical and Economic Review. Gene therapies for sickle cell disease: Evidence report. Boston: ICER; 2023 Jul 13.
14	Lovocel	Sickle cell disease	ICER	Institute for Clinical and Economic Review. Gene therapies for sickle cell disease: Evidence report. Boston: ICER; 2023 Jul 13.
15	Luxturna	IRD	NICE	National Institute for Health and Care Excellence. Voretigene neparovec for treating inherited retinal dystrophies caused by RPE65 gene mutations: Highly specialised technology guidance. London: National Institute for Health and Care Excellence (NICE); 2019 Oct 9.
16	Luxturna	IRD	ICER	Institute for Clinical and Economic Review. Voretigene Neparovec for Biallelic RPE65-Mediated Retinal Disease: Effectiveness and Value. Final evidence report. Boston: ICER; 2018 Feb 14.
17	Luxturna	IRD	CADTH	Pharmacoeconomic Report for voretigene neparovec (Luxturna). Ottawa (ON): Canadian Agency for Drugs and Technologies in Health (CADTH); 2021 Jan.
18	Luxturna	IRD	HAS	Transparency Committee Summary. Luxturna (voretigene neparovec). Paris: Haute Autorité de Santé (HAS). 2019 Apr.
19	Luxturna	IRD	G-BA	Benefit assessment of medicinal products with new active ingredients according to section 35a SGB V: Voretigene neparovec. Gemeinsamer bundesausschuss (G-BA). 2019 Oct 17.
20	Luxturna	IRD	AEMPS	Informe de Posicionamiento Terapéutico de Voretigene neparovec (Luxturna) para distrofia retiniana asociada a la mutacion RPE65 bialelica: AEMPS. 2021 Jun 25.
21	Zolgensma	SMA	NICE	National Institute for Health and Care Excellence. Onasemnogene abeparovec for treating spinal muscular atrophy: Highly specialised technology guidance. London: National Institute for Health and Care Excellence (NICE); 2021 Jul 7.
22	Zolgensma	SMA	ICER	Institute for Clinical and Economic Review. Spinraza® and Zolgensma® for Spinal Muscular Atrophy: Effectiveness and Value: Evidence report. Boston: ICER; 2019 May 24.
23	Zolgensma	SMA	CADTH	Pharmacoeconomic Report for Onasemnogene abeparovec (Zolgensma). Ottawa (ON): Canadian Agency for Drugs and Technologies in Health (CADTH); 2021 May.
24	Zolgensma	SMA	HAS	Avis économique. Zolgensma (Onasemnogene abeparovec). Paris: Haute Autorité de Santé (HAS). 2020 Dec 15.

25	Zolgensma	SMA	G-BA	Bewertung von Arzneimitteln für seltene Leiden nach § 35a Absatz 1 Satz 11 i. V. m. Kapitel 5 § 12 Nr. 1 Satz 2 Verfo Wirkstoff: Onasemnogene abeparvovec. 2020 Oct 1.
26	Zolgensma	SMA	AIFA	Technical Report. Zolgensma (Onasemnogene abeparvovec): Italian Medicines Agency (AIFA). 2021 Jul 12.
27	Zolgensma	SMA	AEMPS	Informe de Posicionamiento Terapéutico de Onasemnogene abeparvovec (Zolgensma®) en Atrofia Muscular Espinal: AEMPS. 2022 Feb 01.
28	Hemgenix	Hemophilia B	ICER	Institute for Clinical and Economic Review. Gene Therapy for Hemophilia B and An Update on Gene Therapy for Hemophilia A: Effectiveness and Value: Final evidence report. Boston: ICER; 2022 Dec 22.
29	Hemgenix	Hemophilia B	HAS	Advice on medications. Etranacogene dezaparvovec. Paris: Haute Autorité de Santé (HAS). 2023 Aug 30.
30	Hemgenix	Hemophilia B	G-BA	Bewertung von Arzneimitteln für seltene Leiden nach § 35a Absatz 1 Satz 11 i. V. m. Kapitel 5 § 12 Nr. 1 Satz 2 Verfo Wirkstoff: Etranacogen Dezaparvovec. 2023 Aug 1.
31	Roctavian	Hemophilia A	ICER	Institute for Clinical and Economic Review. Gene Therapy for Hemophilia B and An Update on Gene Therapy for Hemophilia A: Effectiveness and Value: Final evidence report. Boston: ICER; 2022 Dec 22.
32	Roctavian	Hemophilia A	HAS	Advice on medications. Valoctocogene roxaparvovec. Paris: Haute Autorité de Santé (HAS). 2023 Sep 06.
33	Roctavian	Hemophilia A	G-BA	Benefit assessment of medicinal products with new active ingredients according to section 35a SGB V: valoctocogen roxaparvovec /BMN 270 (ROCTAVIAN). Gemeinsamer bundesausschuss (G-BA). 2022 Dec 15.
34	Roctavian	Hemophilia A	AIFA	VALUTAZIONE DELL'INNOVATIVITA. Roctavian (valoctocogen roxaparvovec): Italian Medicines Agency (AIFA). 2023 Feb.
35	Upstaza	AADC	NICE	National Institute for Health and Care Excellence. Eladocagene exuparvovec for treating aromatic L-amino acid decarboxylase deficiency: Highly specialised technology guidance. London: National Institute for Health and Care Excellence (NICE); 2023 Apr 19.
36	Upstaza	AADC	HAS	Opinion on medicinal products. Eladocagene exuparvovec. Paris: Haute Autorité de Santé (HAS). 2022 Dec 7.
37	Upstaza	AADC	G-BA	Benefit assessment of medicinal products with new active ingredients according to section 35a SGB V: Eladocagene exuparvovec (aromatic-L-amino acid decarboxylase (AADC) deficiency, >18 months. Gemeinsamer bundesausschuss (G-BA). 2023 Feb 02.

38	Upstaza	AADC	AIFA	VALUTAZIONE DELL'INNOVATIVITA. Upstaza (Eladocagene exuparvovec): Italian Medicines Agency (AIFA). 2023 May.
39	Upstaza	AADC	AEMPS	Informe de Posicionamiento Terapéutico de eladocagen exuparvovec (Upstaza®) en pacientes con deficiencia de L-aminoacido descarboxilasa con fenotipo grave: AEMPS. 2023 Aug 10.

Abbreviations: AADC: Aromatic L-Amino Acid Decarboxylase, ADA-SCID: Adenosine Deaminase Deficiency Severe Combined Immunodeficiency, AEMPS: Agencia Española de Medicamentos y Productos Sanitarios, AIFA: Agenzia Italiana del Farmaco, CADTH: Canadian Agency for Drugs and Technologies in Health, G-BA: Gemeinsamer bundesausschuss, HAS: Haute Autorité de Santé, ICER: Institute for Clinical and Economic Review, IRD: inherited retinal dystrophy, MLD: metachromatic leukodystrophy, NICE: National Institute for Health and Care Excellence, SCD: sickle cell disease, SMA: spinal muscular atrophy, TDT: Transfusion-dependent beta-thalassemia.