AAV gene therapy cheat sheet

Philipp Be

ESGCT 2026 Edition

Today (October 2026)

	ascend ABL inc.	AAV1 LPL^{S447X}	AAV2 hRPE65	AAV9 SMN	AAV5 Factor VIII	AAV2 Dopa-Decarboxylase	AAV5 Factor IX	AAVrh74 microDystrophin	AAVrh74 Factor IX	AAV9 SMN	Dual AAV1 OTOF	AAV8 G6PC	AAV9 SGSH (R169Q)
		~~Glybera~~ 2012 EMA	Luxturna 2017 FDA 2018 EMA	Zolgensma 2019 2020	~~Roctavian~~ 2023 2022	Upstaza/*Kebilidi* 2024 2022	Hemgenix 2022 2023	Elevidys 2023	~~Beqvez~~ 2024 2024	Itvisma 2025 2026 „IT-Zolgensma“	Otarmeni 2026	Genglycos 2026	Fayuvi 2026
		uniQure	NOVARTIS Spark	NOVARTIS avexis	BIOMARIN	PTC	uniQure CSL Behring	SAREPTA	Pfizer Spark	NOVARTIS avexis	REGENERON	ultragenyx	ultragenyx
Stability	Room°C	8 h	4 h	8 h	10 h	6 h	24 h	24 h	24 h	5 h	8 h	24h after dilution	24h
	2-8 °C	8 hours	not intended	14 days	3 days	not intended	24 months	14 days	1 day	14 days	1 day	12 days	12 days
	-20°C < -60°C	*18 months*	36 months	24 months	36 months	5 years	not intended	12 months	unknown?	unknown?	unknown?	unknown?	unknown?
Formulation	Concentration	3 x E12 vg/mL	5 x E12 vg/mL	2 x E13 vg/mL	2 x E13 vg/mL	5.6 x E11 vg/mL	1 x E13 vg/mL	1.33 x E13 vg/mL	1 x E13 vg/mL	4 x E13 vg/mL	3 x E13 vg/mL	3 x E13 vg/mL	1 x E13 vg/mL
	Excipients	137 mM NaCl 50 mg/mL Sucrose 2.7 mM KCl 12 mM Phosphate (pH 7.4)	180 mM NaCl 10 mM Phosphate (pH 7.3) 0.001% Poloxamer 188	200 mM NaCl 20 mM TRIS pH 8 1 mM $MgCl_2$ 0.005% Poloxamer 188	140 mM NaCl 20 mg/mL Mannitol 10 mM Phosphate (pH 6.9-7.8) 0.2% Poloxamer 188	337 mM NaCl 3 mM KCl 10 mM Phosphate (pH 6.9) 0.001% Poloxamer 188	137 mM NaCl 50 mg/mL Sucrose 0.2 mg/mL KCl Phosphate (pH 7.1) 0.22 mg/mL Polysorbate 20	200 mM NaCl 20 mM TRIS pH 8 1 mM $MgCl_2$ 0.001% Poloxamer 188	180 mM NaCl 10 mM Phos (pH 7) 0.001% Poloxamer 188	200 mM NaCl 20 mM TRIS pH 8 1 mM $MgCl_2$ 0.005% Poloxamer 188	180 mM NaCl 5% Sucrose 10 mM NaPhos (pH 7.3) 0.001% Poloxamer 188	200 mM NaCl 1 mM $MgCl_2$ * 6 H_2O 20 mM TRIS (pH 7.5-8.5) 0.01% Poloxamer 188	200 mM NaCl 1 mM $MgCl_2$ * 6 H_2O 20 mM TRIS (pH 8.0) 0.001% Poloxamer 188
	Container Type	Glass	AAV Cyclic olefin polymer Diluent	Crystal Zenith®	Cyclic olefin polymer	Type I glass	Type I glass	Cyclic olefin polymer	„Plastic“ vial „plastic“ snap fit cap		2 mL vial	8 mL vial	8 mL vial
	Extractable Vol	1 mL	0.5 mL 1.7 mL	5.5 mL 8.3 mL	8 mL	0.5 mL	10 mL	10 mL	1 mL	3 mL	0.63 mL	2 mL	2 mL
Administration	Route	intramuscular (legs)	subretinal	intravenous	intravenuous	intraparenchymal (brain)	intravenuous	intravenuous	intravenuous	intrathecal	intracochlear	intravenuous	intravenuous
	Dosage	1 x E12 vg/kg	1.5 x E11 vg/eye	1.1 x E14 vg/kg	6 x E13 vg/kg	1.8 x E11 vg total dose	2 x E13 vg/kg	< 70 kg: 1.33 x E14 vg/kg > 70 kg: 9.31 x E15 vg	5 x E11 vg/kg	1.2 x E14 vg	7.2 x E12 vg per ear	1 x E13 vg/kg	3 x E13 vg/kg
	Dose volume (70 kg patient)	24 mL, 47 x 0.5 mL injected	0.3 mL/eye	110 mL (20 kg patient)	210 mL, 27 vials 1-4 mL/min, 2-5 h	0.320 mL as 4 x 0.08 mL infusions 0.003 mL/min, 0.18 mL/h	140 mL ad 500 mL in 0.9% NaCl 8 mL/min, 500 mL/h	20 kg = 200 mL, 20 vials < 10 mL/kg/h (1-2 hours)	3.5 mL ad 200 mL in 0.9% NaCl + 0.25% HSA 3.33 mL/min, 200 mL/h	3 mL for all Bolus injection (1-2 min)	0.24 mL per ear	24 mL, 12 vials ad 250 mL in 0.9 % NaCl	60 mL, 30 vials for 20 kg patient no dilution
	Pre-existing Antibodies	N/A	N/A	anti-AAV9 < 1:50	No anti-AAV5 antibodies *via* FDA-approved test: AAV5 DetectCDx by ARUP Laboratories	N/A	No test required ?!?!	anti-AAVrh74 < 1:400	No anti-AAVrh74 antibodies *via* FDA-approved test: nAbCyte Anti-AAVRh74var HB-FE Assay by LabCorp	anti-AAV9 < 1:50	N/A	No anti-AAV8 antibodies.	Recommended at total binding anti-AAV9 ≥ 1:100

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