

IQANOVA ATLAS — ASMD/Xenpozyme QSP v2.0

Document D2 — References Table

Vancouver style. R-numbered references cited in the manuscript and supporting documents.

Ref ID	Authors	Year	Title	Journal / Source
R1	Wasserstein MP et al.	2022	Olipudase alfa for treatment of acid sphingomyelinase deficiency (ASMD): safety and efficacy in adults treated for 30 months.	Genet Med 24(7):1425-1436
R2	Diaz GA et al.	2024	Long-term safety and efficacy of olipudase alfa in pediatric patients with ASMD.	Mol Genet Metab 142:108441
R3	Schuchman EH, Desnick RJ.	2017	Types A and B Niemann-Pick disease.	Mol Genet Metab 120(1-2):27-33
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R5	Vellodi A et al.	1999	Outcome of enzyme replacement therapy in lysosomal storage disorders.	JIMD 22(7):810-819
R6	Kuchar L et al.	2017	Quantitation of plasma lysosphingomyelin: applications to ASMD.	Anal Biochem 525:73-77
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R9	EMA Product Information.	2024	Xenpozyme (olipudase alfa) summary of product characteristics.	EMA/CHMP/2024
R10	Wasserstein MP et al.	2018	Acid sphingomyelinase deficiency: prevalence and characterisation of an underdiagnosed condition.	Genet Med 20(5):512-518
R11	ICH M15.	2024	General principles for model-informed drug development.	ICH harmonised guideline
R12	ASME V&V40-2018.	2018	Assessing credibility of computational modelling and	ASME

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R13	FDA.	2023	General principles of software validation; final guidance for industry and FDA staff.	FDA Guidance
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R19	Raue A et al.	2009	Structural and practical identifiability analysis of partially observed dynamical models by exploiting the profile likelihood.	Bioinformatics 25:1923-1929
R20	Komorowski M et al.	2011	Sensitivity, robustness and identifiability in stochastic chemical kinetics models.	PNAS 108:8645-8650
R21	Ribba B et al.	2017	Methodologies for QSP modelling.	CPT:PSP 6:496-498
R22	Friedrich CM.	2016	A model qualification method for mechanistic physiological QSP models.	CPT:PSP 5:43-53
R23	Bender BC et al.	2014	A population pharmacokinetic-pharmacodynamic modelling and simulation framework.	Clin Pharmacokinet 53:577-590
R24	Mould DR, Upton RN.	2013	Basic concepts in population modelling, simulation, and model-based drug development.	CPT:PSP 2:e38
R25	Allerheiligen SR.	2014	Impact of MIDD on regulatory decisions.	Clin Pharmacol Ther 95:582-584
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R27	Olivier BG, Bergmann FT.	2018	SBML Level 3 Package: Hierarchical Model Composition.	J Integr Bioinform 15:20170027
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R39	Mager DE et al.	2009	Quantitative pharmacology and physiologically-based pharmacokinetics.	Pharm Res 26:1771-1781