




# MANUFACTURER PERSPECTIVE

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Long-Term Tracking of Clinical Effectiveness, Safety, and Value of Gene Therapies and Implications for EU Joint Clinical Assessment: Multistakeholder Perspectives from Patients, **Manufacturers**, and Payers



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## DISCLOSURES

# LONG-TERM DATA TRACKING REQUIRES COMMITMENT ACROSS PATIENTS, PATIENT REPRESENTATIVES, PROVIDERS, PAYERS, REGULATORS, AND MANUFACTURERS<sup>1</sup>

- Background:
  - Value frameworks, HTA challenge refer to ISPOR EU 2022<sup>1,2</sup>
  - Office of Health Economics Scorecard at ISPOR EU 2023 (QR code)<sup>3</sup>







- Evidence needs of gene therapies for EU joint clinical assessment (JCA)

	 Endpoints	 Standardization	 Monitoring	 Reimbursement
Established Conditions				
Less known conditions				
Key points				

1. Faulkner E. *Value Health*. 2019;22(6):627-641. doi:10.1016/j.jval.2019.04.1911  
2. Besley et al. OHE (2022) Health Technology Assessment of Gene Therapies: Are our methods fit for purpose?  
3. Besley et al. ISPOR EU 2023 Poster # HTA329

# MULTI-FACTORS CONSIDERATIONS FOR LONG-TERM TRACKING OF CLINICAL EFFECTIVENESS, SAFETY, AND VALUE OF TECHNOLOGIES

	 <b>Endpoints</b>	 <b>Standardization</b>	 <b>Monitoring</b>	 <b>Reimbursement</b>
<b>Established Conditions</b>	Factor levels vs. Annualized bleeding rate (ABR) <sup>1</sup>	Recognized Consistent SOC <sup>2</sup>	Many <i>heterogenous</i> treatment centers <sup>2</sup>	Hub & Spoke
<b>Less known conditions</b>	North Star Ambulatory Assessment (NSAA) <sup>4</sup> vs. tomorrow	Next surrogate endpoint... SOC varies across Europe <sup>5</sup>	<ul style="list-style-type: none"> <li>Needs Development</li> <li>Lack of consistent follow up</li> </ul>	Cross-border healthcare?
<b>Key points</b>	Disease and treatments evolution	Intricate evidence generation elements post-approval	<ul style="list-style-type: none"> <li>Maintain long-term follow-up</li> <li>Consistent &amp; rigorous quality site training</li> </ul>	Universal long-term data tracking tools required; archetype fit for purpose reimbursement systems
<b>Ideas</b>				

ABR=annualized bleeding rate; DMD=Duchenne muscular dystrophy; dz=disease; NSSA=north star ambulatory assessment; SOC=standard of care; tx=treatment

1. Keipert C. *Clin Transl Sci.* 2020;13(6):1127-1136. doi:10.1111/cts.12794





2. Windyga J. *The Adv Hematol.* 2022;13:doi:10.1177/20406207221088462

3. Thornburg CD. *Biodrugs* 2023;37(5):595-606. doi:10.1007/s40259-023-00615-4

4. Servais L, Camino E, Clement A, et al *Digit Biomark.* 2021;5(2):183-190. doi:10.1159/000517411

5. Vry J., *J Neuromuscul Dis.* 2016;3(4):517-527. doi:10.3233/JND-160185

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