




Regulatory Strategy and CMC Consulting for Ultra-Rare Disease Cell and Gene Therapies

We are a science-driven consulting boutique with a passion for the patient and expertise in accelerating the development of ultra-rare disease cell and gene therapies. We help you overcome two of the key technical challenges in bringing these therapies to patients in need:

1. Developing a cost-effective manufacturing process via our CMC expertise
2. Providing regulatory guidance and strategic support to ensure your therapy leverages the most efficient pathway against the backdrop of an ever-changing regulatory landscape

WHO WE HELP

-  Preclinical developers of ultra-rare disease CGT treatments
-  Academic groups, non-profits, NGOs, and foundations engaged in early-stage rare disease discovery and development
-  Ultra-rare disease developers in need of regulatory guidance within a dynamically-changing regulatory landscape



8000

Genetic rare diseases
identified globally



95%

Genetic rare diseases
currently have no FDA
approved treatments

Let's partner
and change
the future for
the better



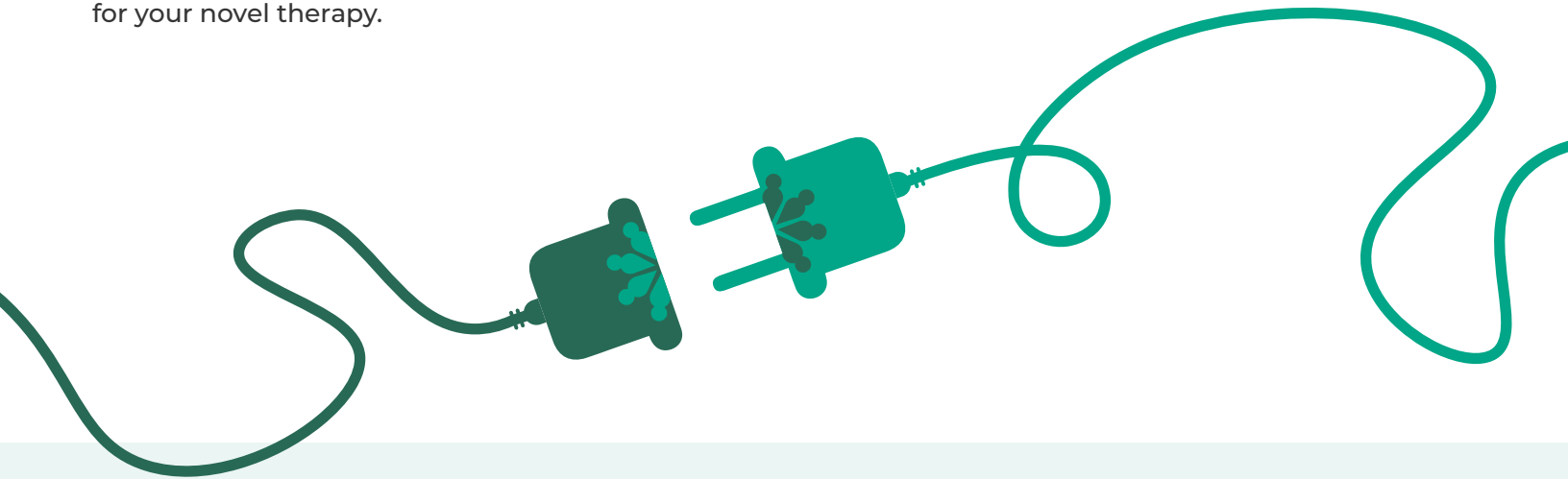
CMC TAILORED TO ULTRA-RARE DISEASE

In traditional biopharma therapeutic development, companies may take years to develop a process, perform engineering runs, then clinical runs, then validation runs. However, in the ultra-rare paradigm, you may only have funding and material for one or two batches. TKD Solutions brings decades of CMC expertise to help you perform high-quality pilot non-GMP runs that are process-representative to efficiently identify Critical Process Attributes and Critical Quality Attributes, laying the CMC foundation for your novel therapy.



REGULATORY EXPERTISE FOR ULTRA-RARE DISEASE

From accelerated approvals to plausible mechanism pathways, TKD Solutions is immersed in the latest regulatory approaches to help bring treatments to patients in need – quickly. We bring this expertise to academic groups, foundations, and preclinical stage companies to give them the best opportunity to bring their treatments forward in a timely manner.



Check out some of our recent CGT contributions in the following publications:

- [1] Eichler, F., Cataltepe, O.I., Daci, R. *et al.* Dual-vector rAAVrh8 gene therapy for GM2 gangliosidosis: a phase 1/2 trial. *Nat Med* 31, 2927–2935 (2025). <https://doi.org/10.1038/s41591-025-03822-4>
- [2] Duenas, A., Smith-Moore, C., Risk Assessment and Comparability Analysis of Adherent Versus Suspension Cultures for Gene Therapy Manufacturing (2025). <https://tkdsolns.com/download/>
- [3] Pier, K., Ingram, L., A Quality by Design Approach to Overcoming the Bottlenecks in Gene Therapy Manufacturing (2024). <https://tkdsolns.com/download/>
- [4] Barrett, David *et al.* Overcoming barriers to commercially pre-viable gene and cell therapies for rare and ultra-rare diseases. *Mol. Ther.*, Volume 33, Issue 11, 5316 - 5326 (2025). <https://doi.org/10.1016/j.ymthe.2025.09.049>

