RareMoon

ORPHAN DRUG REGULATORY SERVICES

PROJECTS RELATED TO GENE & CELL THERAPY*

TYPE OF PRODUCT	INTENDED USE	SCOPE OF WORK
AAV gene therapy Gene editing therapy Gene transfer therapy Enzyme replacement therapy (ERT) Mesenchymal stem cell therapy	Genetic metabolic diseases Lysosomal Storage Disorders Neuromuscular dystrophies Oncology (inc. solid tumors and blood cancers) Dermatological diseases Hematological diseases	***FDA*** Type A, B, C Meetings including pre-IND, EOP1/2, and BTICM Breakthrough Designation Fast Track Orphan Drug Designation IND/CTA Rare Pediatric Designation/Priority Review
		EMA Scientific Advice Pediatric Investigational Plan (PIP) Orphan Drug Designation
		Other IND/CTA/NDA (ICH) GAP Analysis Epidemiology assessment Benchmark analysis EU National Scientific Advice Regulatory Positioning

* Not reflective of experiences prior to RareMoon.