RareMoon

ORPHAN DRUG REGULATORY SERVICES

# PROJECTS RELATED TO PEDIATRIC MEDICINES*

| TYPE OF PRODUCT | INTENDED USE | SCOPE OF WORK |
|---|---|---|
| AAV gene therapy<br>Gene editing therapy<br>Gene transfer technology<br>Small molecules/peptides<br>Nanomedicines | Inherited metabolic diseases<br>Lysosomal Storage Disorders<br>Neuromuscular diseases<br>Muscular Dystrophies<br>Oncology (inc. solid tumors and blood cancers)<br>Dermatological diseases<br>Hematological diseases | ***FDA***<br>Type A, B, C Meetings including pre-IND, EOP1/2, and BTICM<br>Breakthrough Designation<br>Fast Track<br>Orphan Drug Designation<br>IND/CTA<br>Rare Pediatric Designations |
| | | ***EMA***<br>Early Interaction<br>Scientific Advice<br>Pediatric Investigational Plan (PIP)<br>Orphan Drug Designation |
| | | ***Other***<br>IND/CTA/NDA (ICH) GAP Analysis<br>Benchmark analysis<br>Regulatory Roadmap |

* Not reflective of experiences prior to RareMoon.