

Imaging In Rare Disease Trials

Perceptive is a leading provider of proven medical imaging services with extensive experience in musculoskeletal (MSK) endpoint assessments. When tackling the many challenges involved in MSK trial imaging, global clinical trial sponsors repeatedly turn to Perceptive for the right experience, people, processes, and technology to drive their MSK development program's success.

Here we review how Perceptive is uniquely positioned to deliver reliable imaging data to support your pediatric and adult rare disease clinical development programs.

Perceptive has experience in supporting various pediatric and adult rare indications affecting the musculoskeletal system such as Gaucher's disease, Osteogenesis Imperfecta, Pompe disease, Duchenne Muscular Dystrophy, Spinal Muscular Atrophy (SMA), Achondroplasia and Fibrodysplasia Ossificans Progressive (FOP).

We work closely with the client and expert readers/ KOLs to define the endpoints and design appropriate eCRFs to capture efficacy and safety endpoints to support decisions employing various modalities including but not restricted to X-ray, US, MRI, DXA, CT etc.

We tailor the image acquisition guidelines to ensure adequate coverage and appropriate views and implementation of scanning protocols. Toward this end, our experienced technical staff work closely with the sites to review protocol implementation, phantom scan acquisitions as appropriate, and tailored training. Given the nature of rare diseases and the unique image assessments involved, Perceptive is well positioned to provide guidance as well as work closely with relevant experts from the field as needed.

Expertise you can Rely on

Gain access to Perceptive's MSK scientists, who deliver therapeutic and technical expertise in imaging modalities, protocol design, and standardizing image acquisition across global investigative sites to drive your clinical development program's success.

Some of the criteria/assessments we have supported for rare disease trials are:

- ▼ Quantification of heterotrophic bone ossification
- ▼ MRI based Bone Marrow Burden score (BMB score)
- ▼ X-ray and MRI based for growth plate and bone age assessments in pediatric populations
- ▼ DXA and MRI for bone and body composition indices
- ▼ Fracture and bone abnormality assessments

Engaging KOLs and Expert Readers

Our flexibility in choosing MSK-experienced readers enables you to utilize virtually any KOL as an expert reader. We collaborate with you to identify the most reliable readers and optimal read model for your trial, balancing their read volumes, and ensuring low inter-reader variability to drive the highest quality imaging reads for your study.

Global Operational Support

Perceptive's operational model enables our client delivery teams to anticipate your study needs and scale our services to deliver quick and reliable continuity of service with efficient setup of sites, dedicated teams for QC support, and reduced imaging queries to meet your trial timelines.

With deep operational oversight delivered through MSK trial-experienced project managers, Perceptive delivers the flexibility and scalability trial sponsors need during all stages of MSK treatment development, helping them to overcome challenges and confidently tackle unexpected trial changes.

Contact hello@perceptive.com to learn how Perceptive can support your pediatric and adult rare disease clinical development programs.