Ensayos clínicos actualmente en marcha:

1. Patología neuromuscular

- Biogen – Study of Nusinersen (BIIB058) Among Patients With Spinal Muscular Atrophy Who Received Onasemnogene Abeparvovec (RESPOND) – NCT04488133
- Biogen – A Long-Term Extension Study of Nusinersen (BIIB058) Administered at Higher Doses in Participants With Spinal Muscular Atrophy (ONWARD) – NCT04729907
- Novartis – Manejo terapéutico y situación actual de uso de recursos y costes de la AME en España (TREaSURE) – NCT06632730
- Quince Therapeutics – Evaluate the Neurological Effects of EryDex on Subjects With A-T (NEAT) – NCT06193200
- IntraBio Ltd. – A Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Safety and Efficacy of IB1001 in Subjects With Ataxia-Telangiectasia – NCT03759678
- Reata / Biogen – Safety, Pharmacokinetics and Efficacy of Omaveloxolone in Children and Adolescents With Friedreich's Ataxia (BRAVE) – NCT06953583
- Roche – A Study to Investigate the Safety and Efficacy of RO7204239 in Combination With Risdiplam in Participants With Spinal Muscular Atrophy (MANATEE) – NCT05115110
- NMD Pharma – Safety and Efficacy of NMD670 in Ambulatory Adult Patients With Type 3 Spinal Muscular Atrophy – NCT05794139

2. Inmunodeficiencias

- 200075 (ZOSTER-047) A Phase II, randomised, open-label, multicentre study to assess the reactogenicity, safety and immunogenicity of GSK's paediatric Herpes Zoster subunit candidate vaccine (PED-HZ/su) when administered intramuscularly on a two-dose schedule to immunocompromised paediatric renal transplant recipients from 1 to 17 years of age. Inicio 2019.IP HULP: T del Rosal, IC: C Calvo, A Méndez, C Fernández Camblor, L Espinosa, M. Melgosa, A. Alonso.
- IISP 61381 Fase 3. "Immunogenicity of 9-valent HPV vaccine in immunocompromised children". PI: T Sainz / T Rosal. SI: A Mendez, C Calvo, P Rodríguez, S Alcolea. Funding: Merck, Sharp & Dohme. Inicio: Mayo 2023

Colaboración con ensayos clínicos internacionales:

- Terapia Génica en déficit de ADA. Londres. Great Ormond. IP Claire Both.
- Terapia Génica en Wiskott Aldrich: Milán. San Rafaelle Scientific Institute IP: Francesca Ferrua

3. Enfermedades autoinmunes

- I4V-MC-JAHU: Phase III. A Randomized, Double-Blind, Placebo-Controlled, Withdrawal, Safety and Efficacy Study of Oral Baricitinib in Patients from 2 Years to Less Than 18 Years Old with Systemic Juvenile Idiopathic Arthritis (SJIA).
- -I4V-MC-JAHV: A Randomized, Double-Blind, Placebo-Controlled, Withdrawal, Safety and Efficacy Study of Oral Baricitinib in Patients from 2 Years to Less Than 18 Years Old with Juvenile Idiopathic Arthritis (JIA). Phase 3

- -I4V-MC-JAHW: Phase III. Open-label, Active-Controlled, Safety, and Efficacy Study of Oral Baricitinib in Patients from 2 Years to Less Than 18 Years Old with Active Juvenile Idiopathic Arthritis-Associated Uveitis or Chronic Anterior Antinuclear Antibody-Positive Uveitis.
- I4V-MC-JAHX: A Phase 3 Multicenter Study to Evaluate the Long-Term Safety and Efficacy of Baricitinib in Patients from 1 Year to < 18 Years of Age with Juvenile Idiopathic Arthritis (JIA).
- M15-340: Phase I. An Open-Label Multiple-Dose Study to Evaluate the Pharmacokinetics, Safety, and Tolerability of Upadacitinib in Pediatric Subjects with Polyarticular Course Juvenile Idiopathic Arthritis
- M15-340: Phase I. An Open-Label Multiple-Dose Study to Evaluate the Pharmacokinetics, Safety, and Tolerability of Upadacitinib in Pediatric Subjects with Polyarticular Course Juvenile Idiopathic Arthritis
- A3921145: A long Term, Open Label Follow Up Study of Tofacitinib for Treatment of Juvenile Idiopathic Arthritis (JIA)
- NCT05039619. A Study to Evaluate the Efficacy, Safety, and Pharmacokinetics of Obinutuzumab in Adolescents With Active Class III or IV Lupus Nephritis and the Safety and PK of Obinutuzumab in Pediatric Participants (POSTERITY)
- NCT05835310. An Efficacy and Safety Study of Intravenous Anifrolumab to Treat Systemic Lupus Erythematosus in Pediatric Participants (BLOSSOM)

4. Enfermedades endocrino-genéticas

- Prospective clinical assessment study in children with achondroplasia: the propel trial, Código QBGJ398-001, sponsorized QED Therapeutics, Inc. NCT 03732820
- PROPEL OLE: Estudio de extensión de fase II, abierto, a largo plazo de infigratinib, un inhibidor selectivo de la tirosina quinasa de los receptores del factor de crecimiento fibroblástico 1-3 (FGFR 1-3), en niños con acondroplasia. NCT05145010
- BMN 111-603: ACORN: Estudio multicéntrico, no intervencionista para evaluar la seguridad a largo plazo en pacientes con acondroplasia tratada con Voxzogo® (vosoritida). EUPAS47514
- BMN-111-902 A Multicenter Multinational Observational Study of children with Hypochondroplasia. NCT06212947
- ALXN1850-HPP-305: A Phase 3, Randomized, Double-blinded, Placebo-controlled, Multicenter Study to Evaluate Efficacy and Safety of ALXN1850 Versus Placebo Administered Subcutaneously in Pediatric (2 to < 12 years of age) Participants with Hypophosphatasia Who Have Not Received Previous Treatment with Asfotase Alfa. NCT06079359