

Study of the Safety and Efficacy of an Adeno-Associated Viral Vector Carrying the SMN Gene After a Single Intravenous Administration of Escalating Doses in Children With Spinal Muscular Atrophy (BLUE-BELL)

NCT05747261

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Biocad
Enrollment	40 participants

Key Eligibility Criteria

Inclusion (6)

- Informed consent form for participation in the study signed by the subject's legal representative;
- Subjects of either sex under the age of 240 days at the time of signing the Information Sheet for the Legal Representative of the Clinical Study Subject with Informed Consent Form;
- A diagnosis of 5q-SMA (homozygous deletion of exon 7 of the SMN1 gene or heterozygous deletion of exon 7 + confirmed point mutation of the SMN1 gene) and 2 or 3 copies of the SMN2 gene established based on molecular genetic testing;
- Subjects with 2 copies of the SMN2 gene can be included in the study both at the presymptomatic stage of the disease and in the presence of SMA symptoms. If symptoms are present, the age of onset of the disease should be up to 180 days from birth.
- Subjects with 3 copies of the SMN2 gene can be included in the study if they have symptoms of SMA type 1 and the disease began before the age of 180 days.

... and 1 more (see full listing online)

Exclusion (17)

- A diagnosis of HIV infection, hepatitis B, hepatitis C, congenital syphilis in the study subject, as well as a documented diagnosis of HIV infection in the study subject's mother. Note: documented hepatitis B and/or hepatitis C and/or syphilis in the mother of a study subject is not an exclusion criterion in this clinical study, provided that standard breastfeeding rules are followed or the subject is not breastfed due to the low risk of transmission of hepatitis B and C viruses and *Treponema pallidum* from mother to child with breast milk;
- Unwillingness of the legal representative to use alternative feeding methods (nasogastric tube, gastrostomy) in case of swallowing disorders and a risk of aspiration;
- Anti-AAV9 antibody titer $\geq 1:50$ determined by ELISA. Note: if a subject's screening anti-AAV9 antibody titer is $\geq 1:50$, the anti-AAV9 antibody titer may be determined again. Subjects with anti-AAV9 antibody titers $\geq 1:50$ in the second test may be included in the study;
- Need for respiratory support for ≥ 16 hours per day or tracheostomy ;
- Treatment with nusinersen, risdiplam, branaplam, onasemnogene abeparvovec or other antisense oligonucleotides/selective SMN2 splicing modifiers or gene therapy drugs for SMN1 transduction or other AAV-based gene therapy drugs regardless of serotype used previously (from birth) or planned for the main study period, i.e., within 12 months after the administration of the investigational product.

... and 12 more (see full listing online)

Locations (7 total)

1. State Institution Republican Scientific and Practical Center "Mother and Child", Minsk, Belarus
Federal State Autonomous Educational Institution of Higher Education "N.I. Pirogov Russian National Research Medical University", Ministry of Health of the Russian Federation, Moscow, Russia
Federal State Autonomous Educational Institution of Higher Education "N.I. Pirogov Russian National Research Medical University", Ministry of Health of the Russian Federation, Moscow, Russia

... and 4 more locations

<https://clinicaltrials.gov/study/NCT05747261>

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