Detailed description of the subject of the order

Purchase financed from the state budget from the Medical Research Agency as part of the project:

Development of a universal fast-response platform, based on RNA technology, ensuring the national drug and epidemiological safety

Financing contract number: 2021/ABM/05/00004-00

Technical specification:

1. Cell line no. 1: iPSC line derived from a patient with spinal muscular atrophy (SMA) type 1 or type 2. It should carry a homozygous mutation—specifically, a deletion of exons 7 and 8 in the *SMN1* gene—and no more than three copies of the *SMN2* gene (eg. GM24468, Coriell). The mother of the donor should be a carrier of the SMN1 mutation but should not exhibit any clinical symptoms.
2. Cell line no. 2: iPSC line derived from a carrier of the SMN1 mutation who does not exhibit clinical symptoms of spinal muscular atrophy (SMA). It should carry a heterozygous mutation—specifically, a deletion of exons 7 and 8 in the *SMN1* gene—and two copies of the *SMN2* gene (eg. GM24474, Coriell).
3. Cell line no. 3: fibroblast cell line derived from a patient with spinal muscular atrophy (SMA) type 1 of Polish/German origin. The patient should be homozygous for a deletion of exons 7 and 8 in the *SMN1* gene and should carry three copies of the *SMN2* (eg. GM09677, Coriell).
4. Cell line no. 4: fibroblast cell line derived from a patient with spinal muscular atrophy (SMA) type 2. The patient should be homozygous for a deletion of exons 7 and 8 in the *SMN1* gene and should carry three copies of the *SMN2*. The donor of cell line no. 4 should be biologically related to the donor of control cell line no. 5: child–mother relationship (eg. GM03813, Coriell). The mother of the donor should be a carrier of the SMN1 mutation but should not exhibit any clinical symptoms.
5. Cell line no. 5: fibroblast cell line derived from a healthy control individual without symptoms of spinal muscular atrophy (SMA). The donor should be heterozygous for a deletion of exons 7 and 8 in the *SMN1* gene and should carry five copies of the *SMN2* gene. The donor of cell line no. 5 should be biologically related to the affected donor of cell line no. 4 (mother–child relationship). (eg. GM03814, Coriell).