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Ethical challenges in pediatric neurology clinical trials

Clinical trials in the pediatric neurology patient population are very common in the last decade. There are clinical trials reflecting rare forms of epilepsy and neuromuscular diseases. Few examples are provided below:

Dravet syndrome (DS) or severe myoclonic epilepsy in infant is one of the most well-known disorders of the epileptic encephalopathies. DS is a highly treatment-resistant and refractory epilepsy syndrome. Establishment of a seizure free condition in affected children, even with anticonvulsant drug polypharmacy is extremely rare. Lennox-Gastaut Syndrome (LGS) is a rare epileptic encephalopathy. Onset of LGS usually occurs before age 11, with a peak between 3 and 5 years of age. Nearly all LGS patients have treatment-resistant, lifelong epilepsy, with a poor prognosis. Duchenne muscular dystrophy (DMD) is a disabling and life-threatening X-linked genetic disorder affecting males. Boys with DMD develop progressive proximal muscle weakness that leads to deterioration of ambulation, wheelchair dependency, and eventual respiratory and cardiac failure. Spinal muscular atrophy (SMA) is an autosomal recessive neuromuscular disease resulting in atrophy of the voluntary muscles of the limbs and trunk. It is the most common genetic cause of infant mortality, and a major cause of childhood morbidity in the U.S.

Clinical trials in pediatric neurology rare diseases are testing new investigational drugs (ids). There is still an ethical dilemma if the new treatment will be better than the standard of care. There are number of unknown risks. Patient's enrollment can be very challenging. The commitment from the families is huge. Most of the clinical trials start with the double-blind, placebo-controlled design. Families have to agree on the possibility for their child to be randomized in the placebo arm. Those clinical trials might have very frequent study visits which might cause financial burden. Lot of work needs to be done to address the ethical challenges.

Speaker Biography

Vesna Popovska has graduated from medical school in 1988 in Skopje, Macedonia. She completed her residency in obstetrics & gynaecology in 1998 in Macedonia. She moved to Canada in 1999 and joined the Division of Maternal Fetal Medicine (MFM) at BC Women's Hospital, where she built, established and lead the entire research program as a program manager. In 2005, she was recruited by the neurosciences program at BC Children's Hospital, as a senior research manager. She developed and led the program, becoming its director in 2016. She is involved in the strategic planning, implementation and evaluation of the program activities and the management of multiple complex projects. She is responsible for recruitment, supervision and evaluation of all research staff at neurosciences program. She continues to lead the program, develops collaborative relationship among universities, industry partners and Clinical Research Organizations (CROs) to support and advance patient-oriented research.

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