# Single Patient Study Support

**BACKGROUND**

Over the last 2 years, there have been 23 single patient studies (SPS) in Canada, approximately half of those are in pediatrics. The Health Canada criteria for an SPS is: the patient has a serious or life-threatening condition, are not eligible for, or have exhausted all other treatment options, or have no other way to access a particular treatment. Although designed to allow for treatment under these circumstances, an SPS is still considered a clinical trial and therefore is subjected to follow the rigorous requirements of Part C, Division 5 "Drugs for Clinical Trials Involving Human Subjects" of the Food and Drug Act Regulations. The advantages to conducting an SPS over applying to the Special Access Program (SAP) include: regulated access to a product when other pathways are no longer an option, and the ability to collect data to support the advancement of knowledge in regard to the investigational product.

**ISSUE**

As a result of an SPS being considered a regulated clinical trial, a study sponsor is also required. The sponsor obligations are lengthy and comprehensive and include, among others, submitting a Clinical Trial Application (CTA), obtaining ethics approval and informed consent, implementing a plan for adverse event reporting and study monitoring, management of essential documents, acquiring appropriate certifications and training, and adhering to pharmacy and labelling requirements. For pediatric oncology SPS', C17 has taken on the role of sponsor, providing study teams and participants with preparatory packages and support. For non-oncology pediatric SPS', such as those for rare diseases, there remains a gap in available guidance, resources, and assistance.

**SOLUTION OFFERING**

One of MICYRN's core objectives is to provide safe, efficient, national infrastructure support to increase access to high-quality clinical trials, particularly in the rare disease space. To that end, MICYRN is building capacity to support the following services for non-oncology single patient studies in pediatrics:

- Support the development of the study protocol, with required Health Canada elements, including access to methodology experts (adaptive, cross over, Bayesian, sequential trial design)
- Prepare the CTA submission and assist in obtaining required product information, in acceptable format, from the manufacturer(s) in support of the application (i.e., letter of access)
- Provide guidance and support for adverse event reporting to Health Canada
- Provide ongoing Health Canada support for any amendments/notifications, etc.
- Discuss monitoring OR cross training of monitors for high-risk trials
- Assist in providing appropriate training and certifications

Support will be offered either directly by the MICYRN team or facilitated by the MICYRN team through contracted service agreements with our partners at a subsidized cost. If you or an investigator at your institution is seeking support for a non-oncology SPS, please contact Breanne Stewart at breanne1@ualberta.ca.