

Web Accessible Population Pharmacokinetics Service - Hemophilia

Principal Investigator: Alfonso Iorio, MD, PhD

LAY SUMMARY

The benefit of Hemophilia treatment increases when it is tailored to individual patient characteristics, including how the factor is eliminated from the body, which can be assessed by dosing factor levels over several days. Population pharmacokinetics combines clinical epidemiology, pharmacology and mathematics to allow the same calculation from a few blood samples. Population pharmacokinetics has been used in cancer care and transplantation. We will set up an online system to allow calculation of individual factor disposition for all the available factor concentrates, improving hemophilia care for adults and particularly children. This system will be the first in its kind.

STRUCTURED ABSTRACT

Objective: Setting up a centralized web-accessible service employing population pharmacokinetics methodology to assess clinically relevant pharmacokinetics of factor VIII and IX with the following functionalities: a) the input of patient data, b) automatic estimation of pharmacokinetic (PK) parameters, c) expert validation of the estimation process and d) local reporting of PK parameters.

Methods: The project is streamlined into 10 work units (WUs):

- WU#1 Development of webaccessible user interface to upload anonymized patient data and download population PK analysis reports;
- WU#2 Development of a multilanguage and mobile versions;
- WU#3 Development of administrative interface to allow management of system users and validation of PK estimates;
- WU#4 Development of a Bayesian hierarchical population PK calculator;
- WU#5 Priming of the population PK engine with available PK data;
- WU#6 User testing;
- WU#7 Development of a user guide and interactive tutorial;
- WU#8 Recruitment of pilot centers to test system with real data;
- WU#9 Cumulative analysis of estimates evolution and test of statistical variance;
- WU#10 Release of open webpages reporting efficiency statistics in real time.

Results: The core WUs (#1, #2, #3, #4) will be developed in parallel; and the software will be available in 18 months.

Conclusions: The system will be the first dedicated population PK calculator available on an institutional website, empowering hemophilia treatment by simplifying individual PK assessment. This should result in better care, intended both as more effective prophylaxis and optimization of resource utilization. The system will progressively accrue population and individual PK data for both hemophilia A and B and multiple brands and multiple laboratory test for factor levels, quickly constituting the worldwide widest PK data repository in the field, bringing to Canada a unique knowledge base and expertise. The system will be advertised widely and in different fields (bleeding disorders, knowledge translation, medical informatics, pharmacokinetics, health economics and health policy).