

MOTION 426

AMENDED MOTION

That “in the opinion of the House, the government should respond specifically to the challenges faced by Canadians with rare diseases and disorders, in collaboration with provinces and territories (P/Ts) and stakeholders by:

- (a) examining options for defining serious rare diseases;
- (b) examining options, including the possible creation of a specific fund, to improve access to rare disease treatments, building on the recent work undertaken by federal and provincial/territorial governments under the National Pharmaceuticals Strategy;
- (c) considering the establishment of a multi-stakeholder advisory body, including the Common Drug Review, treaters and patients, to recommend treatment access for life-threatening or serious rare disorders, based on scientific standards and social values;
- (d) exploring options to consider national and international expert advice in developing criteria for treating patients based on scientific evidence and patient impact, and to link these activities with ongoing post-market monitoring of real world drug safety and effectiveness;
- (e) considering options to encourage research and development into treatments for rare diseases and other unmet health needs;
- (f) considering internationally accepted standards for conduct of clinical trials in rare disorders appropriate for the challenges inherent to very small patient populations;
- (g) considering how Health Canada's work on a progressive licensing framework could provide appropriate support to the design of clinical trials for very small patient populations and appropriate review of evidence submitted from these trials; and,
- (h) reporting the progress accomplished to the House within 12 months”.

ORIGINAL MOTION

That, in the opinion of the House, the government should respond specifically to the challenges faced by Canadians with rare diseases and disorders, and the initiative put forward by the Canadian Organization for Rare Disorders by: (a) establishing the definition for serious rare disorders as those with a prevalence of less than 1 in 2000 Canadians; (b) examining the feasibility of a national “Chance for Life Fund” equivalent to 2% of the total annual public drug expenditure to be designated for therapies for rare disorders; (c) considering the establishment of a multi-stakeholder advisory body, including treaters and patients, to recommend treatment access for life-threatening or serious rare disorders, based on scientific standards and social values; (d) considering the establishment of centres of reference for specific rare disorders, comprised of national and international experts, who will develop criteria for treating patients based on scientific evidence and patient impact and provide on-going surveillance into the real-world safety and effectiveness of these treatments on individual and group basis; (e) considering options to provide incentives through orphan drug regulation and policy, to assure Canadian organizations and researchers are motivated to conduct research and development into treatments for rare and neglected disorders; (f) supporting internationally accepted standards for conduct of clinical trials in rare disorders appropriate for the challenges inherent to very small patient populations; (g) considering ensuring that Health Canada’s progressive licensing framework provide appropriate support to the design of clinical trials for very small patient populations and appropriate review of evidence submitted from these trials; and (h) reporting the progress accomplished to the House within six months.