Challenges associated with access to recently developed hemophilia treatments in routine care: perspectives of healthcare professionals

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Supplemental Materials

IPSG Access Survey

General Information

- Email address
- Job profile (e.g., adult hematologist, hemophilia treatment centre medical director)
- Name of hemophilia treatment centre
- What type of patients are followed at your HTC?
    - Pediatrics (< or =18 years)
    - Adults (>18 years)
    - Adults and pediatrics

Survey Questions

1. In what country are you currently practicing or working: _____

2. Who covers the expenses of hemophilia care in your country?
    a. Public insurance (government)
    b. Private insurance companies
    c. Patient only
    d. Other only
        i. Please specify: _____
    e. Combination of any of the above (please estimate proportion of coverage)
        i. Public insurance: _____ %
        ii. Private insurance: _____ %
        iii. Patient: _____ %
        iv. Other: _____ %
        v. I don't know: _____ %

3. In your country, does the price influence access to treatment?
    a. Yes
    b. No
    c. I don't know

4. In your country, is access to hemostatic agents:
    a. The same for both adults and children < 18 years
    b. Greater for adults
    c. Greater for children < 18 years
    d. I don't know

5. Comments on product restrictions (e.g., Pegylated FVIII/IX products may not be approved by a national body such as the FDA/EMA for use in boys with hemophilia < 12 years of age): _____

6. Please select one answer for each of the following statements below:

*Enhanced half-life (EHL) clotting factor concentrates, non-factor treatment, gene therapy

	Strongly Disagree	Disagree	Neither Agree nor Disagree	Agree	Strongly Agree
There are existing situations in routine care where economic considerations impact your therapeutic choices					
Access to innovative therapeutics* is limited to certain indications					
EHL-FVIII					
EHL-FIX					
Non-factor hemostatic therapies					
Gene therapy					
There are haemophilia drugs you would like to prescribe but they are not available					
You are adequately informed about the relation of costs and benefits of your therapeutic recommendations					
Patients/families (e.g., parents/guardians) are currently more involved in the shared decision making regarding the use of innovative therapies for treatment					

7. If you agree that access is limited to certain indications, please provide any ethical issues that may influence how you prescribe factor (e.g., cost): _____

The following list of questions relate. To documents to be submitted to payers or Health Technology Assessment bodies (HTA) for reimbursement of new therapies and treatments for haemophilia.

8. Is there a national HTA (Health Technology Assessment) body or equivalent in your country whose responsibility is to evaluate new drugs and treatments? (e.g., CADTH)
    a. Yes
    b. No
    c. I don't know

9. Please indicate the name of the HTA body in your country/region (select all that apply).
    a. Government
    b. Institution
    c. Other
    d. I don't know

10. If Government, please specify the name of the body: _____
11. If Institution, please specify the name of the body: _____
12. If Other, please specify the name of the body: _____

13. Does the HTA body in your country have a well-defined and transparent process in terms of methodological requirements and assessment methods for recommending/approving reimbursement of hemophilia treatments?
    a. Yes
    b. No
    c. I don't know

14. What type of evidence is the strongest driver in benefit assessment for reimbursement recommendations/decisions in your country? Please rank the items below from 1 to 4 (1 – Most Important and 4 – Least Important).

	1	2	3	4
Randomized controlled trials (RCT)				
Real world data (RWD) (e.g., registry, longitudinal observational studies)				
Systematic literature review				
Other				

15. If "Other" type of evidence, please specify: _____

16. What type of health economic aspects are part of the HTA?
    a. Cost-effectiveness analyses
    b. Budget impact analysis
    c. I don't know

17. Are budget impact analyses of new hemophilia treatments requested by payers of hemostatic therapies in your country?
    a. Yes
    b. No
    c. I don't know

18. Is there a hemophilia patient organization in your country/region?
    a. Yes
    b. No
    c. I don't know

19. Does this organization lobby for access?
    a. Yes
    b. No
    c. I don't know

20. Does the patient organization have a vote at the decision-making table regarding the funding for a hemostatic agent that has regulatory approval (i.e., Health Canada/FDA/EMA, etc.) and support from a formal Health Technology Assessment or equivalent?
    a. Yes
    b. No
    c. I don't know

21. Are there any comments you would like to share with us that are relevant to access to hemostatic therapies for the use and prevention of bleeding in persons with hemophilia and other severe inherited bleeding disorders registered and followed in your hemophilia treatment centre: _____

22. Please provide any comments and/or suggested revisions you may have regarding the IPSG Access Survey: _____