

DEPARTMENT OF BIOLOGICAL STANDARDISATION, OMCL
NETWORK & HEALTHCARE (DBO)

GR/nko

Working document, with no legally binding status,
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(listed opposite). Level 1

English / Anglais

PA/PH/TS (19) 21 DEF

Strasbourg, May 2020

EUROPEAN COMMITTEE (PARTIAL AGREEMENT) ON BLOOD TRANSFUSION (CD-P-TS)

RECOMMENDATIONS ON HAEMOPHILIA TREATMENT

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RECOMMENDATIONS ON HAEMOPHILIA TREATMENT

Recommendation 1

Prophylactic treatment should be available to all haemophilia patients, with or without inhibitors, and easy access to specialised physiotherapy should be provided.

Recommendation 2

With increased treatment options, appropriate instruments should be developed to personalise treatment regimens for all patients with haemophilia A and/or B.

Recommendation 3

With extended half-life therapies, a minimum trough level of 3-5% should be achieved to preserve joint status.

Recommendation 4

For the post infusion measurement of extended half-life products chromogenic assays should be used.

Recommendation 5

When using non-replacement therapies, some laboratory issues should be considered: for example, for patients on emicizumab, chromogenic assays with human reagents or modified FVIII one-stage clotting assays with calibrators should be used to measure the procoagulant activity. Chromogenic assays with bovine reagents should be used to measure FVIII levels after infusion of FVIII concentrate and in the estimation of FVIII inhibitors.

Recommendation 6

The management of patients with haemophilia, particularly those using non-replacement therapies and gene therapies, should be supervised by Comprehensive Care Centres, such as the certified European Haemophilia Comprehensive Care Centres (EHCCCs), or in countries where the latter are not available, by European Haemophilia Treatment Centres (EHTCs).

Recommendation 7

Post-marketing data collection for the long-term safety and efficacy of all products should be mandatory. Every country should establish a national patient registry for haemophilia and other inherited bleeding disorders, covering all treatment modalities and patient-relevant outcomes.

Recommendation 8

Dedicated data governance, evaluation and reporting should be implemented with adequate sustainable financial support under public control. Core data elements as recommended by the EMA and a minimal dataset for post-registration surveillance should be implemented according to the communication from the ISTH Scientific and Standardization Subcommittee (SSC).

Recommendation 9

Collaboration at the European level should be encouraged, by strengthening and harmonising existing registries in order to facilitate pooling and comprehensive evaluation of data.

Recommendation 10

Data collection should include direct reporting from patients using appropriate electronic industry-independent tools. Its use should be supported by education, user-friendly applications, and positive feedback to the patients.

Recommendation 11

Clinical studies should be performed to provide the best possible evidence needed for regulatory authorities, HTA bodies, academia and healthcare providers.

Recommendation 12

A process to reach better agreement on relevant indicators and methods should be started in order to meet the needs of regulatory authorities, HTA bodies, academia and healthcare providers that have different foci, responsibilities and requirements.

Recommendation 13

A dialogue with agencies and academia should be started in order to reach understanding of relevant outcomes and to ensure comprehensive and consistent reporting.