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EHC Statement on the Discontinuation or Refocusing of Multiple Haemophilia Treatment Programmes

The European Haemophilia Consortium (EHC) acknowledges the recent decisions by several pharmaceutical companies to discontinue or refocus the development or commercialisation of haemophilia therapies since October 2024:

- **Pfizer** will halt the global commercialisation of **fidanacogene elaparvovec-dzkt**, a gene therapy for haemophilia B, citing low demand. Approved in multiple regions, including the European Union, this one-time therapy will no longer be pursued, with Pfizer shifting focus to marstacimab-hncq, an injectable treatment for haemophilia A and B.
- **Sangamo Therapeutics** will regain full development and commercialisation rights to **giroctocogene fitelparvovec**, an investigational haemophilia A gene therapy, after Pfizer's decision to exit the collaboration and terminate the licensing agreement.
- **Spark Therapeutics** has discontinued its **phase 3 study of dirloctocogene samoparvovec** for haemophilia A but will instead focus on developing an enhanced-function Factor VIII variant, with a phase 2b trial expected in 2025. The phase 3 study was stopped before dosing any participants, with no safety concerns cited.
- **Centessa Pharmaceuticals** will stop the global development of **SerpinPC**, a serine protease inhibitor for haemophilia B, despite its favourable safety profile. The company cited changes in the treatment landscape, including a recent FDA approval of a similar product, as the reason for discontinuation.

The EHC recognises that these decisions come at a time when significant unmet needs persist in the bleeding disorders community. While progress has been made, many people with haemophilia (PWH) or other bleeding disorders still experience treatment burdens, breakthrough bleeding, and reduced quality of life. Access to standard prophylaxis remains a challenge in many European countries, further underscoring the need for continued research, innovation, and equitable treatment availability.

The EHC urges pharmaceutical companies, health authorities, and stakeholders to remain committed to advancing treatment options for haemophilia and other bleeding disorders. Investment in research and development must continue to ensure that the needs of the bleeding disorders community are met and that all patients have access to safe, effective and innovative therapies.