



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Factor VIII and factor IX development plans at the Paediatric Committee

Overview

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An agency of the European Union





Progress – development in children

- Factor VIII

- Feb 2013, 86 PTP, >12yrs; Jan 2013 50 PTP <12yrs; Feb 2018, 25 PUP
- Mar 2013, 10 PTP, >12yrs; Sep 2015 50 PTP <12yrs; Sep 2019, 100 PUP
- June 2014, 10 PTP, >12yrs; Mar 2016 50 PTP <12yrs; Dec 2020, 50 PUP
- Jan 2015, 8 PTP, >12yrs; Jun 2015 50 PTP <12yrs; May 2018, 50 PUP
- Oct 2010, 50 PTP, >12yrs; Jul 2014, 20 PUP
- Sep 2011, 17 PTP, >12yrs; Jan 2012 50 PTP <12yrs; Feb 2016, 50 PUP
- Sep 2012, 18 PTP, >12yrs; Nov 2012 50 PTP <12yrs; Dec 2018, 50 PUP
- Dec 2016 50 PTP <12yrs; June 2021, 100 PUP
- Dec 2014 15 PTP >12yrs; Aug 2015 50 PTP <12yrs; Dec 2022, 50 PUP

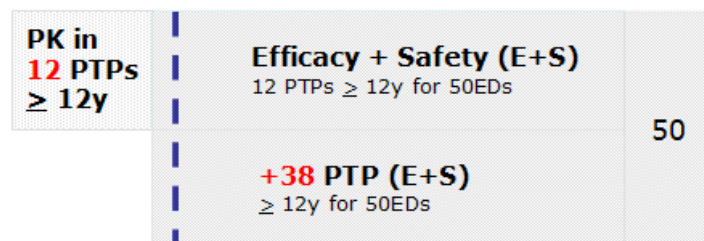
- Factor IX

- July 2012, 20 PTP, >12yrs; June 2015 20 PTP <12yrs; 2019, 40 PUP
- July 2011, 24 PTP, >12yrs; June 2012, 12 PTP, >12yrs; Oct 2014, 50 PTP, >12yrs; Sep 2014 20 PTP <12yrs; June 2017, 20 PUP
- June 2013, 15 PTP, >12yrs; Aug 2014, 20 PTP, <12yrs; Oct 2020, 20 PUP
- Jan 2011, 50 PTP, >12yrs; Jul 2015, 20 PTP, <12yrs; Nov 2019, 40 PUP

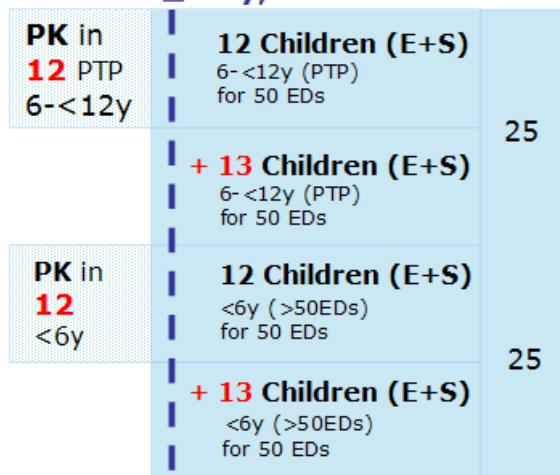


'standard' Paediatric Investigation Plan FVIII

Pre-authorisation



▼ **20 PTP ≥ 12y, 50 ED**



↓ **20pts <12y, 50 ED¹**

Post-authorisation

MA (earliest time point)

PMI:
200 PTP for 100 EDs in total (PTP from pre-authorisation studies can be followed up to 100 EDs, "new" PTPs for 100 EDs; PMI study participants should include at least 60 PTP <12y based on age distribution in haemophiliacs (e.g. Soucie *et al* 1994))

50 PUPs^{2,3} (E+S)
for 50 EDs

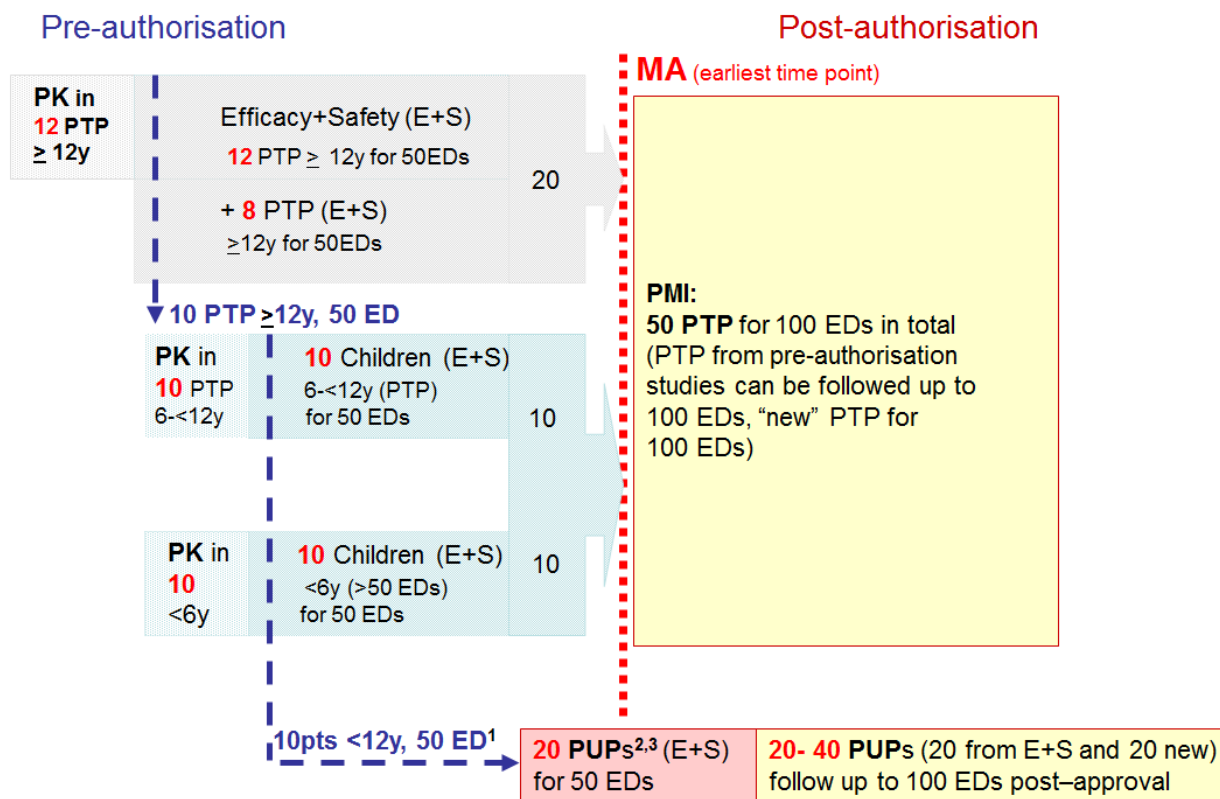
100 PUPs (50 from E+S and 50 new)
follow up to 100EDs post – approval

SmPC for novel products: Indication is restricted until data from 50 PUPs (E+S) are available!

¹ min. 10 patients <6y and pk in children 0-<12y completed
² plasma-derived factor VIII products = case by case
³ completion of clinical study in 50 PUPs not required for initial MAA however for approval of indication in PUPs for novel products



Proposed 'standard' PIP FIX



¹ min. 5 patients <6y and pk in children 0-<12y completed

² plasma-derived factor IX products = case by case

³ completion of clinical study in 20 PUPs not required for initial MAA, however for approval of indication in PUPs for novel products

SmPC of novel products: Indication is restricted until data from 20 PUPs (E+S) are available!