EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Factor VIII and factor IX development plans at the Paediatric Committee

Overview

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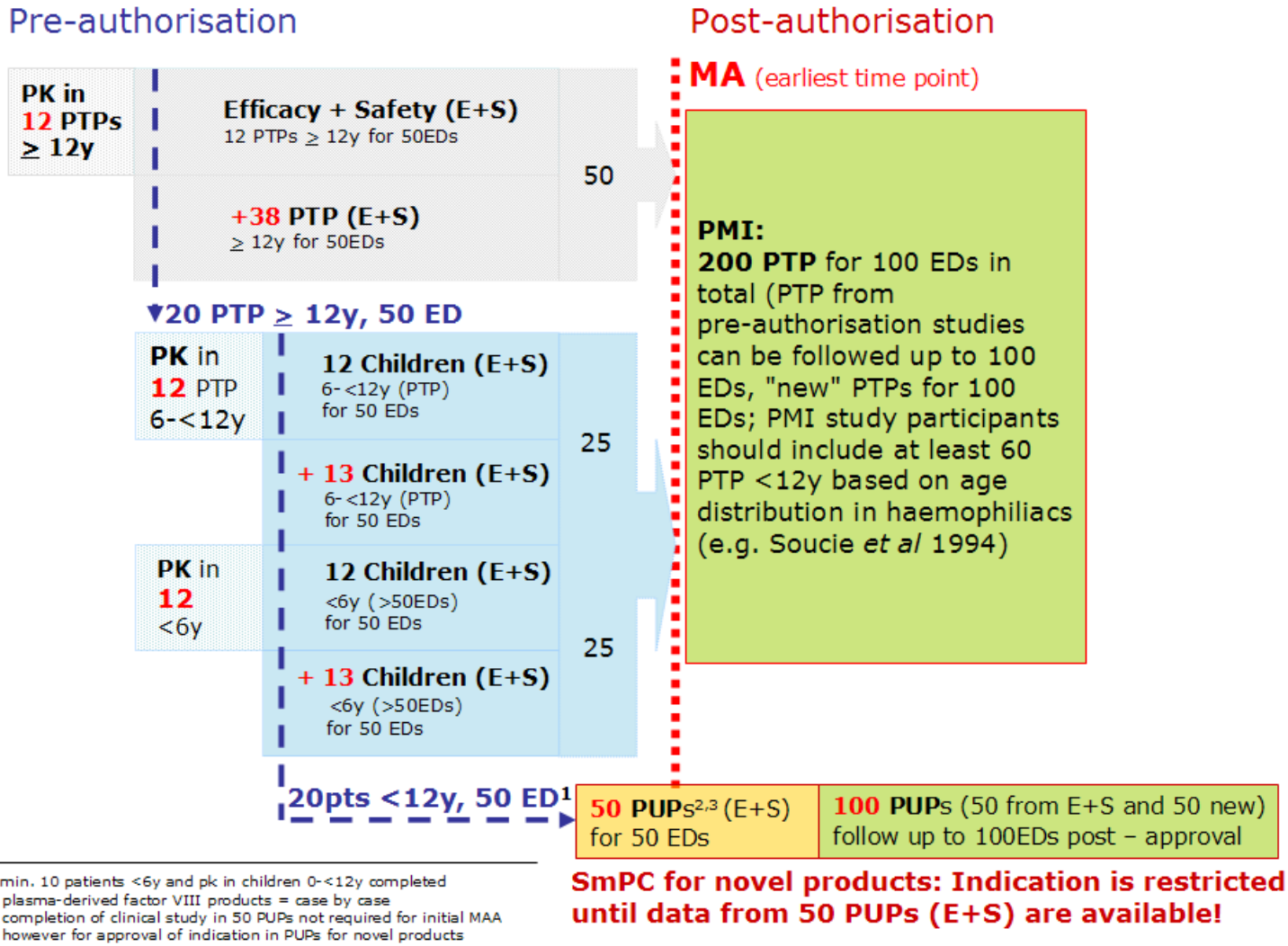
Progress – development in children

- 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

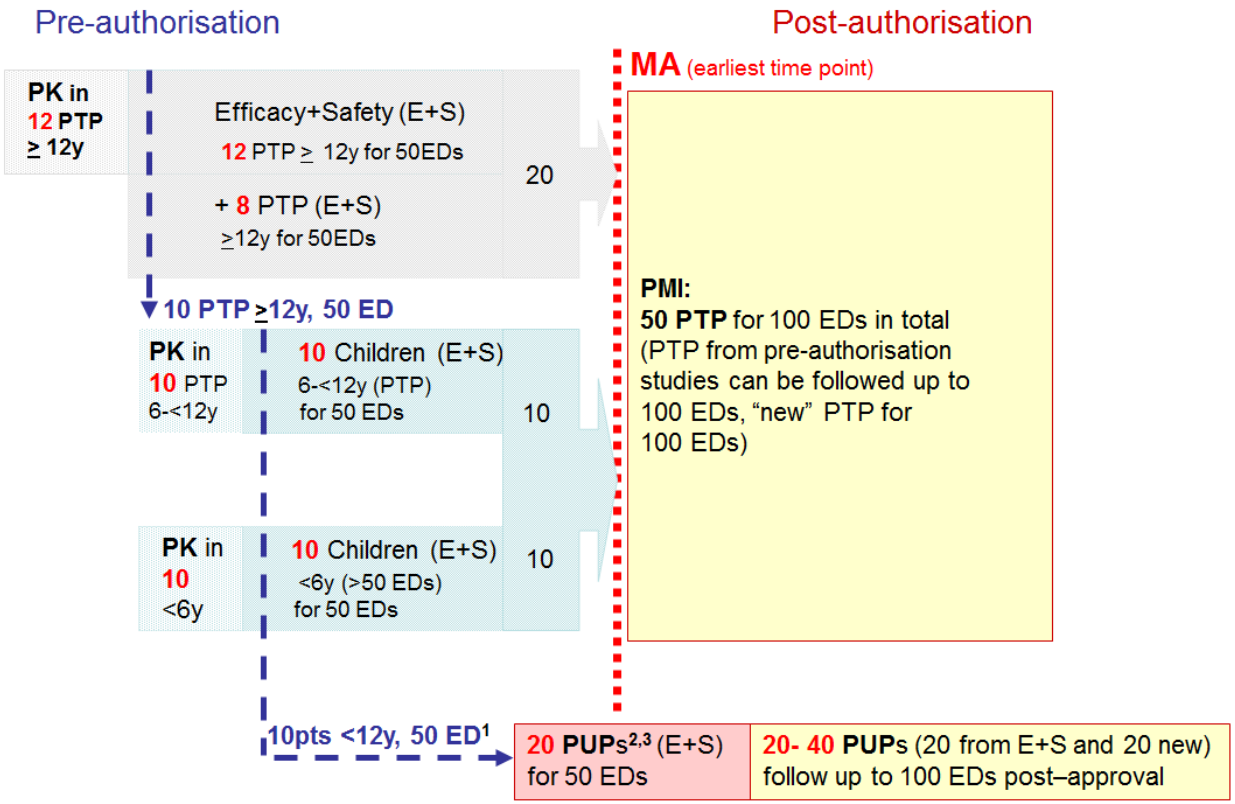
‘standard’ Paediatric Investigation Plan FVIII



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!