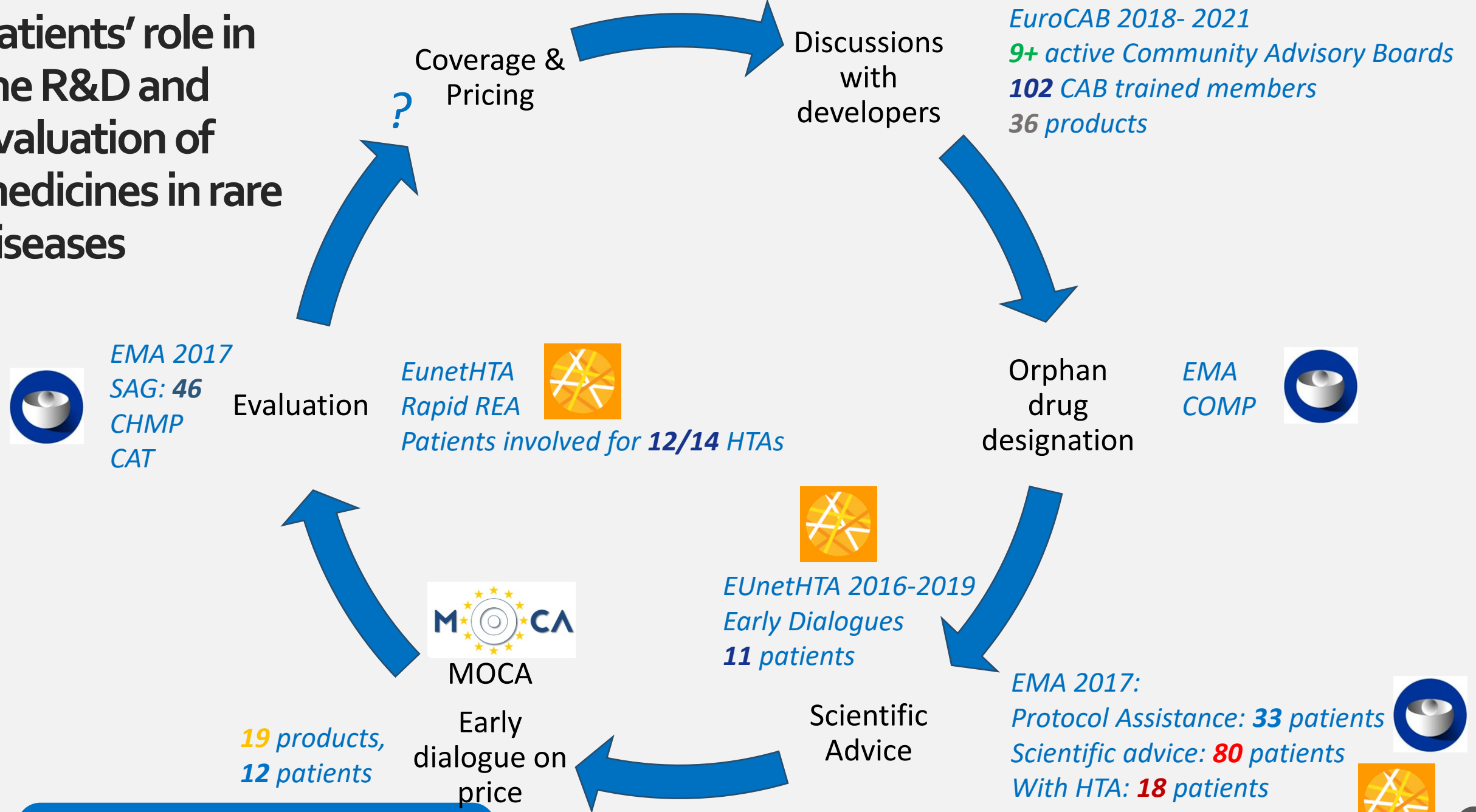




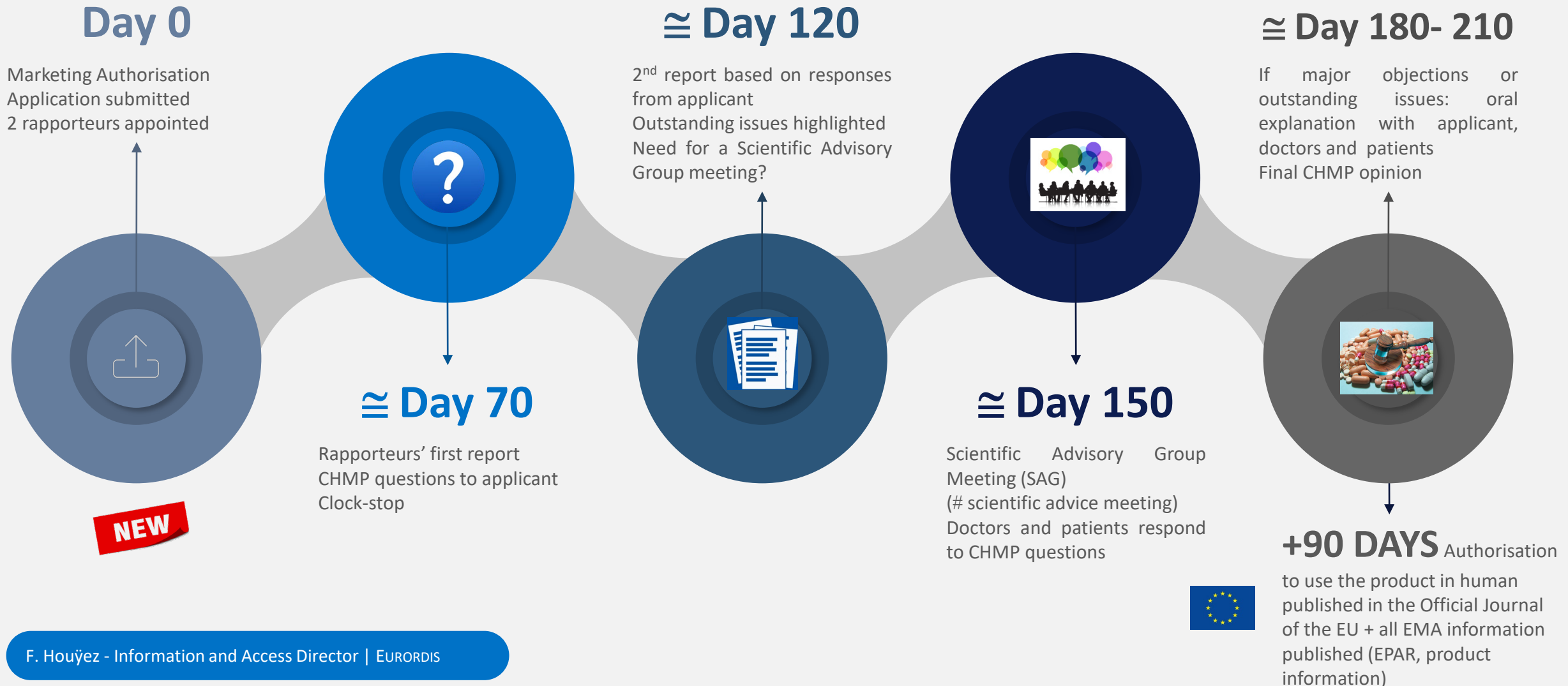
A NEW PILOT @ EMA

KNOW WHAT YOU EXPECT FROM A NEW
MEDICINE

Patients' role in the R&D and evaluation of medicines in rare diseases



Timelines of medicine evaluation at EMA: Committee for Human Medicinal Products



The process

For all submissions of a marketing authorisation application for a rare disease



When rapporteurs appointed

- EMA contacts EURORDIS with a template document
 - Product name, active substance name, disease / indication
 - Questions
 - Deadline (usually 3 weeks)



EURORDIS

- Identifies patients for phone / video conference interviews
 - Orphanet, ERNs, EURORDIS contact database
 - EMA template used as guide for semi-guided interviews
- Explores websites of relevant organisations to search for answers in relation to EMA questions
- Summarises responses in 2 to 3 pages

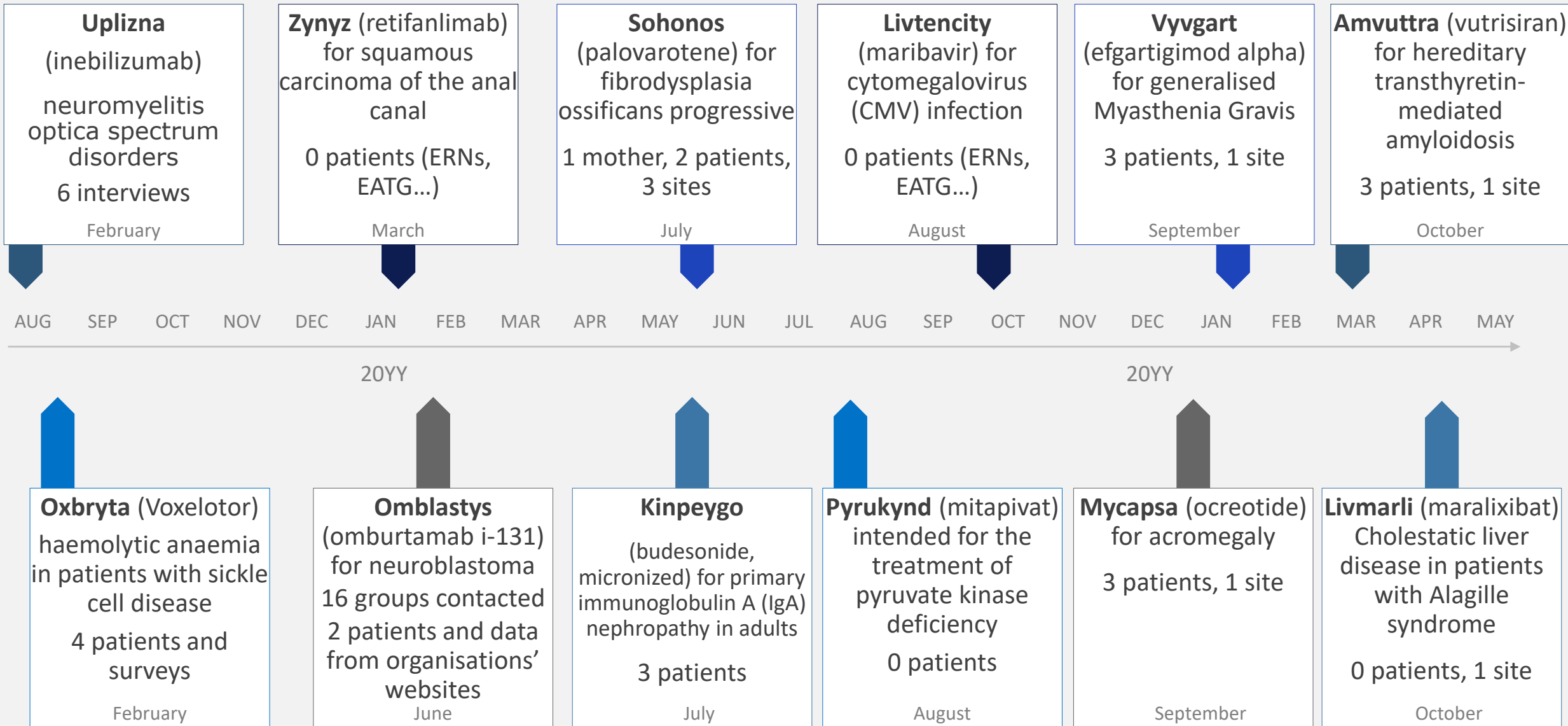


Memo

- Sent back to EMA / rapporteurs
- Shared with patients who were interviewed (usually 2 to 3)
- Sent to Marketing Authorisation Applicant (patients' names not mentioned)

Progress in 2021

27 interviews (1 hour each) for 8 products), and 3 failures / 12 (no report sent back to EMA)



PILOT

- The rapporteurs can start analysing the data having in mind aspects that are important to you

- Know your unmet needs

Which treatment exist for your disease in which countries?

What benefits? What limits?

Highlight if there are differences between groups of patients about these aspects or if these views are generally similar across the condition

- Know the unknown about your disease

Any aspects about the condition or its treatments that you feel are not well-understood or not sufficiently considered

- Your expectations, relevant questions

What do you expect from a new treatment?

And from this one in particular (if you heard from trial participants or CAB discussions)?

Anything you feel is important for EMA to know?

CHMP pilot – early contact with patients



“In writing” procedure via a patient advocate experienced in the work of rapporteurs (“to put yourself in the mind of rapporteurs and of patients”)



Interviews with patients extremely useful
To be considered for discussions on patient engagement in HTA
We should be doing it systematically and earlier in the process



CHMP / EMA are evaluating contributions received. Pilot to last at least one year. Other groups also involved.



You can prepare yourself and organise discussions with members, publish your views
Avoid copy-pasting information from HCP



Houyez F. High Price Medicines and Health Budgets: The Role Patients' and Consumers' Organisations Can Play
European Journal of Health Law. 18 May 2020, Volume 27: Issue 3,309–323.
DOI: <https://doi.org/10.1163/15718093-BJA10008>

THANK YOU

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