'Challenges in patient access to orphan therapies and possible solutions“

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EURORDIS
RARE DISEASES EUROPE

70%

OF PEOPLE WITH RARE DISEASES

wait more than 1 year to get a confirmed diagnosis after coming to medical attention.

It takes on average

5 YEARS

for rare disease patients to get a diagnosis.

THERE ARE OVER

230

ORPHAN MEDICINES

authorised in the EU. The goal is to support support the development of

1,000

NEW THERAPIES

for rare diseases by 2030.

EURORDIS.ORG

Treatment authorisation in Europe

Since 2000

2782 Orphan designations

277 Orphan designations included in authorised indication

244 Authorised OMPs

97 To be used in children

6 Removed from the market

84 Marketed, but no longer "orphans"

To date

154

Products with a marketing authorisation and an orphan status in the European Union

25 October 2023

Science is progressively offer more hope for PLWRD

Beyfortus, the first medicine for the prevention of respiratory syncytial virus (RSV) lower respiratory tract disease in newborns and infants during their first RSV season (when there is a risk of RSV infection in the community).

Breyanzi, a gene therapy for the treatment of adult patients with three subtypes of non-Hodgkin lymphoma (diffuse large B-cell lymphoma (DLBCL), primary mediastinal large B-cell lymphoma (PMBCL) and follicular lymphoma grade 3B (FL3B)), whose cancer has come back or who have not responded to treatment after two or more lines of systemic therapy.

Carvykti, for the treatment of adults with relapsed and refractory multiple myeloma who have received at least three prior therapies and whose cancer has worsened since they received their last treatment.

Ebvallo, for the treatment of Epstein-Barr virus positive post-transplant lymphoproliferative disease. This somatic cell therapy is intended for adult and paediatric patients who develop this malignancy after transplantation as a result of the immunosuppression caused by the medication required to reduce the possibility of rejection of the transplanted organ or bone marrow.

Hemgenix, the first gene therapy for the treatment of severe and moderately severe Haemophilia B in adults, an inherited disorder characterised by an increased bleeding tendency due to a partial or complete deficiency in the activity of factor IX.

Kimmtrak, a monotherapy for treatment of adult patients with a form of eye cancer called uveal melanoma.

Mounjaro, a first-in-class medicine that activates both the GLP-1 and GIP receptors, which leads to improved blood sugar control in adults with type-2 diabetes mellitus.

Roctavian, for the treatment of severe haemophilia A in adults who do not have factor VIII inhibitors (auto-antibodies produced by the immune system which make factor VIII medicines less effective) and no antibodies to adeno-associated virus serotype 5 (AAV5).

Upstaza, the first treatment for adult and paediatric patients with aromatic L-amino acid decarboxylase (AADC) deficiency, an ultra-rare genetic disorder affecting the nervous system.

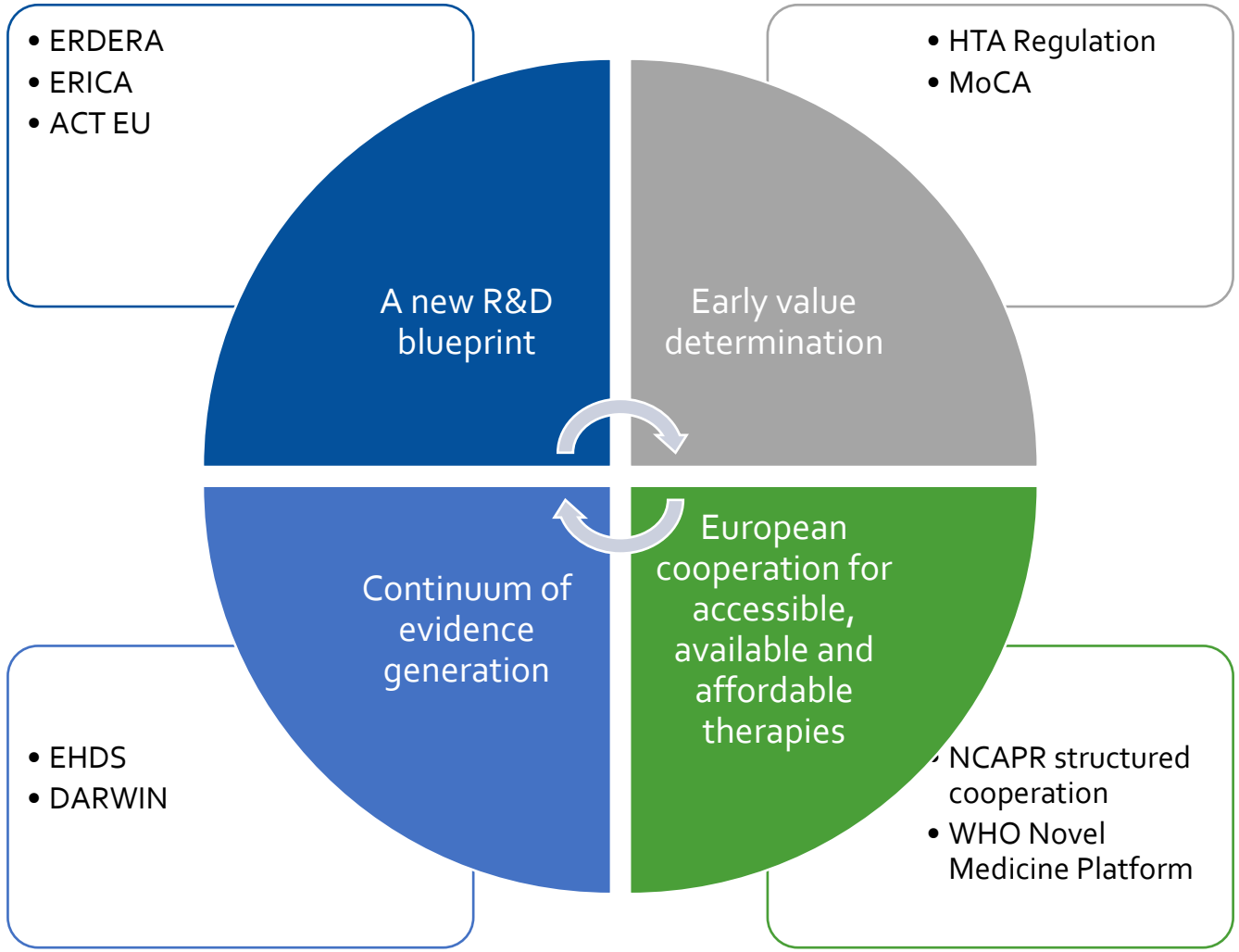
SOURCE: EMA Annual Report 2022
https://www.ema.europa.eu/en/documents/annual-report/2022-annual-report-european-medicines-agency_en.pdf

But …

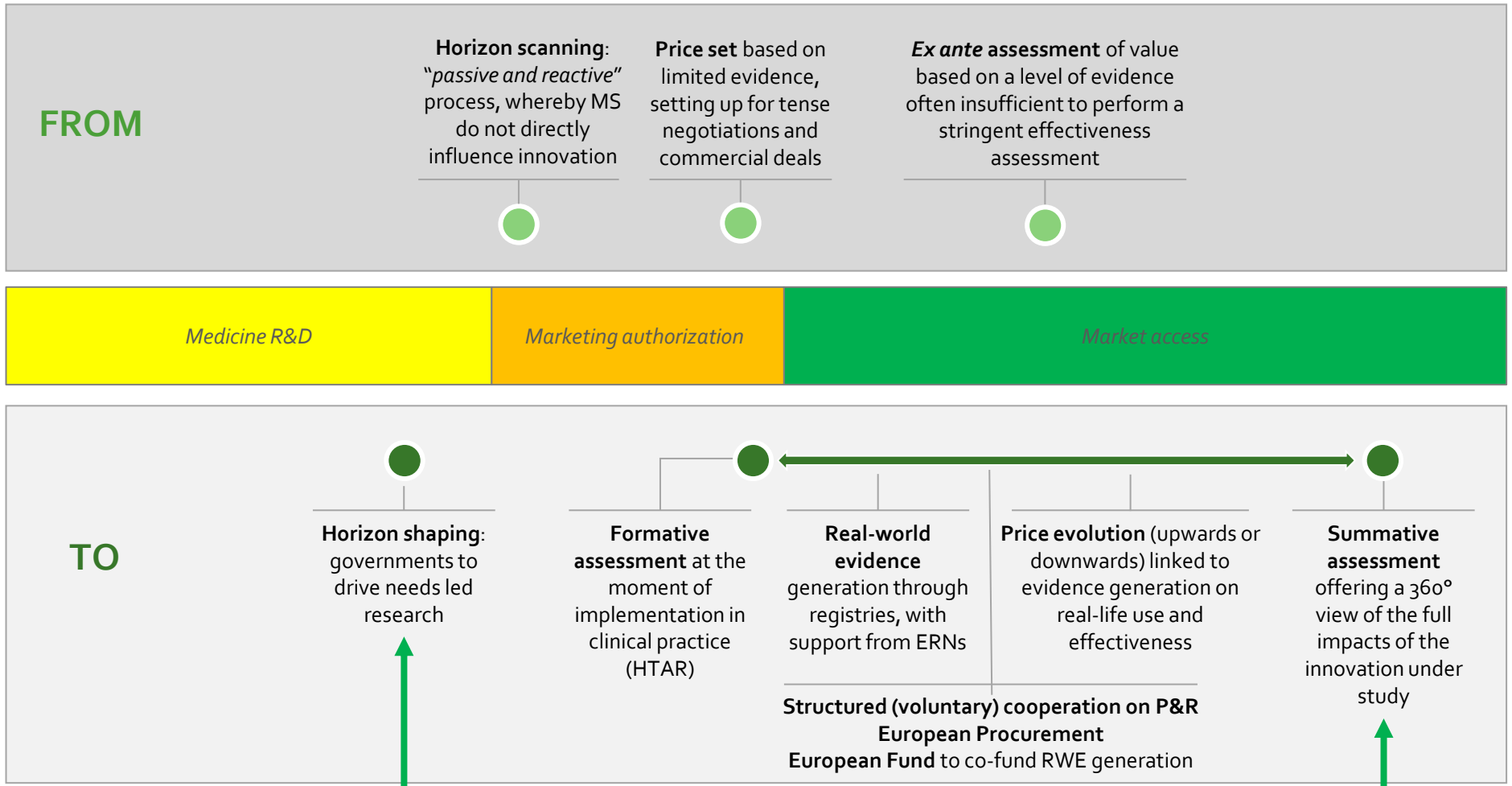
Only 5% had received a transformative treatment approved for the entire European Union, with 69% of rare disease patients having received only symptomatic treatment for their rare disease

22% of people with rare diseases could not get the treatments they needed because it was not available where they live, reflective of the fragmentation of the market across the 27 Member States

A new approach is possible



A new life cycle perspective on access to rare disease therapies



- Status quo **not acceptable**
- **Transparency** of decision processes often lacking
- **Inequalities** due to mismatch of science and economy

- Allow for ***timely*** and ***equitable*** access
- Ensure ***predictability*** of revenues
- Complete continuum of ***evidence generation***

THANK YOU