



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Non-oncological MoA based PIP – Initial Deliberations from the PDCO working group discussions

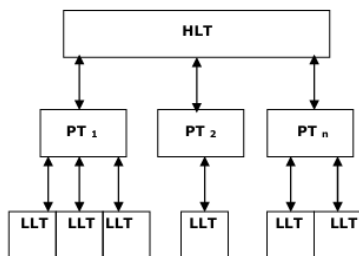
Sabine Scherer on behalf of the PDCO working group (Chrissi Pallidis, Sylvie Benchetrit, Yury Kiselev)





Determination of the PIP condition

- **Currently** the PIP condition for an active compound is defined based on the *Policy on the determination of the condition(s) for a PIP/Waiver*
- The proposed adult indication, mode of action, medical need (e.g off-label use) and MedRA system (High level term) are taken into account



HLT	Motor neurone diseases
PT	Amyotrophic lateral sclerosis
PT	Bulbospinal muscular atrophy congenital
PT	Familial amyotrophic lateral sclerosis
PT	Foramen magnum syndrome
PT	Hereditary motor neurone disease
PT	Lower motor neurone lesion
PT	Monomelic amyotrophy
PT	Motor neurone disease
PT	Progressive bulbar palsy
PT	Progressive muscular atrophy
PT	Pyramidal tract syndrome
PT	Spinal muscular atrophy
PT	Upper motor neurone lesion

- The proposal for the **new pharma legislation** includes a mode of action based approach

Article 75 (Regulation)

Waivers

...the Agency may decide that the production of the information...shall be waived for products or for classes of medicinal products, if there is evidence showing...

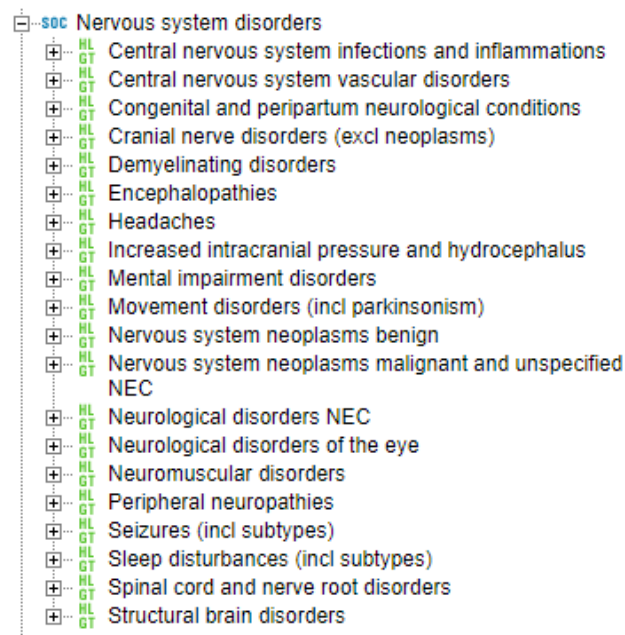
1. (b) that the disease or condition for which the specific medicinal product or class is intended occurs only in adult populations, **unless when the product is directed at a molecular target or due to its mechanism of action on the basis of existing scientific data, is responsible for a different disease or condition in the same therapeutic area in children** than the one for which the specific medicinal product or class of medicinal products is intended for in the adult population;
- Valid for waivers based on disease not occurring only? (2021: 12, 2022: 16, 2023: 4)

	2021	2022	2023
N	12	16	4



Mode of action PIP

- Definition of “*same therapeutic area*”? MedRA System Organ Class? Word condition based on MoA?





Which areas are most likely to profit?

- **Neonatology**
 - A product that is developed for COPD might be useful for the treatment of BPD
 - COPD is on **class waiver list** -> Class waiver list needs to be revised
- **Neurodegenerative diseases**
 - Products developed for Alzheimer disease might be useful for paed. tauopathies, but available scientific data is limited -> **stepwise approach could be used**
 - MoA will be difficult to apply in very rare paed. diseases as the **potential to extrapolation** will be limited due to the lack of an adult program
 - In case of paediatric specific therapeutic need, a specific development according to drug characteristics might be appropriate: e.g. aggressiveness instead of major depressive disorder

-> **further case studies are planned**



Thank you!