LP pro vzácná onemocnění- „orphany“

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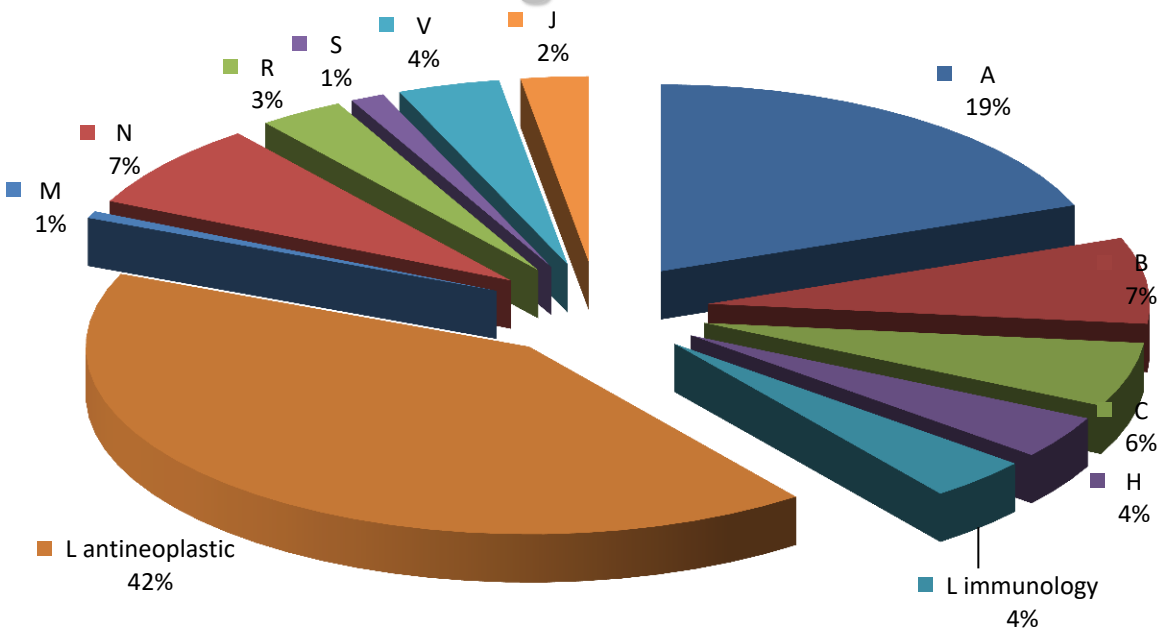
142 initial orphan marketing authorisations and 20 extension of indication granted to date

A Alimentary tract and metabolism
B Haematology
C Cardiovascular system
H Systemic hormonal
J Anti-infectives for systemic use
L Immunology
L Antineoplastic
M Musculo-skeletal system
N Nervous system
R Respiratory system
S Sensory organs
V Various

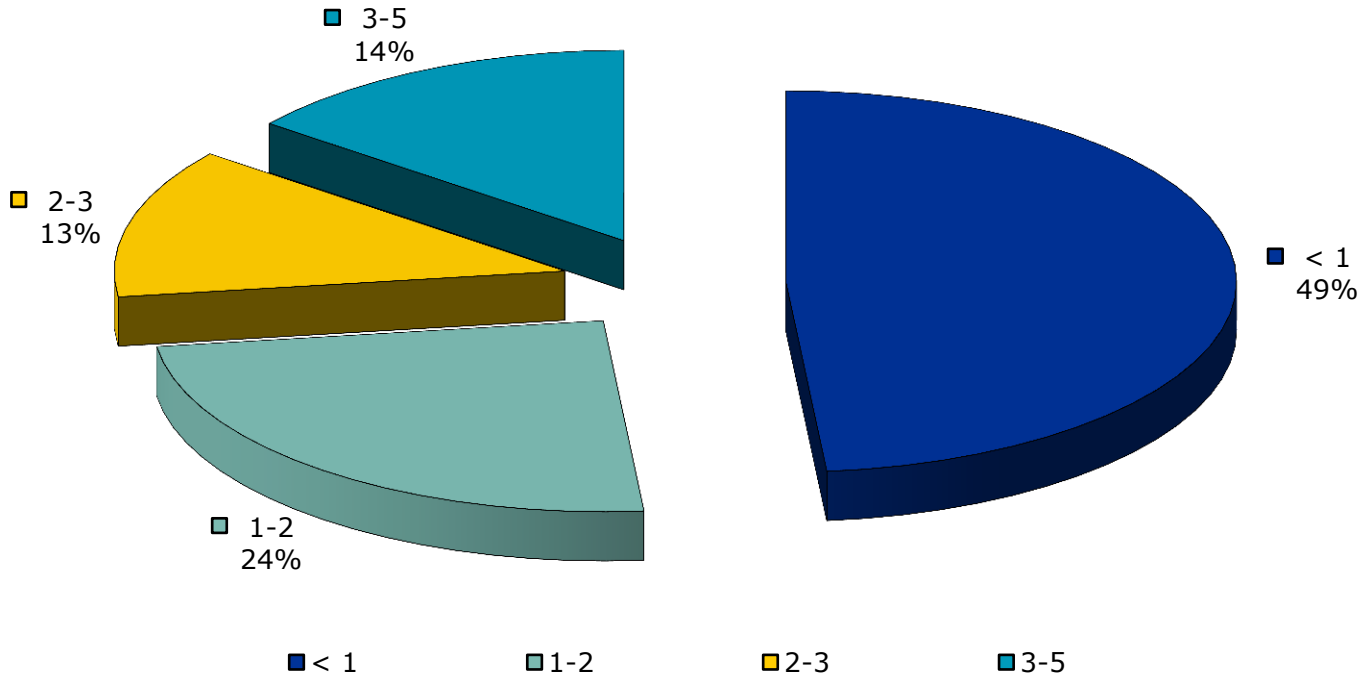


Number of conditions: 111
Active orphan MA: 94
Active extension of indication: 13

Chart includes:
13 authorised extensions of indication
13 withdrawals from the register of orphan medicinal products (including 6 ext. of indication)
5 withdrawals from register medicinal products human use
36 removals of initial MAA from register after expire of the market exclusivity period & 1 ext of indication

Prevalence for orphan marketing authorisations period 2000-2017

Prevalence per 10,000 all orphan products
Including extensions of indication,
withdrawals and products which market exclusivity has expired



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Human medicines highlights 2017

Authorisation of new medicines

Overview of the key figures on the European Medicines Agency's (EMA) recommendations for the authorisation of new medicines in 2017:

92 Positive opinions

35 New active substances

6 Negative opinions

14 Withdrawn applications

2 Advanced therapy medicinal products

19 Orphan medicines*

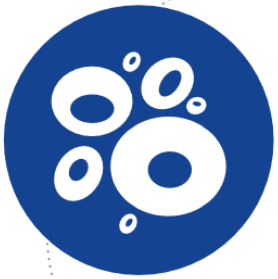
7 Accelerated assessments

3 Conditional marketing authorisations

2 Approval under exceptional circumstances

See more on the new recommendations from page 2.

Medicines recommended for approval



Cancer

- **Axumin**
- **Bavencio**
- **Besponsa**
- Blitzima
- **Fotivda**
- Fulvestrant Mylan
- Herzuma
- Imatinib Teva B.V.
- **Kisqali**
- **Lutathera**
- Mvasi
- Ontruzant
- Pemetrexed Hospira Limited
- Qarziba (previously Dinutuximab beta Apeiron)
- Ritemvia
- Rixathon
- Riximyo
- **Rydapt**
- **Tecentriq**
- **Tookad**
- Tuxella
- **Varuby**
- **Zejula**

Infections

- Daptomycin Hospira
- Darunavir Krka
- Darunavir Krka d.d.
- Efavirenz/Emtricitabine/Tenofovir disoproxil Krka
- Efavirenz/Emtricitabine/Tenofovir disoproxil Mylan
- Efavirenz/Emtricitabine/Tenofovir disoproxil Zentiva
- Emtricitabine/tenofovir disoproxil Krka d.d.
- Entecavir Accord
- Entecavir Mylan
- **Maviret**
- **Prevymis**
- Ritonavir Mylan
- Symtuza
- **Trumenba**
- **Vosevi**

Endocrinology

- Alkindi
- **Crysvita**
- Insulin lispro Sanofi Intrarosa
- Ivabradine Accord
- Miglustat Gen.Orph
- Natpar
- Nitisinone MendeliKABS
- **Ozempic**
- Ucedane
- **Xermelo**
- Yargesa

Immunology/ Rheumatology/ Transplantation

- Amgevita
- Cyltezo
- Erelzi
- Imraldi
- Jylamvo 2mg/ml Oral Solution
- **Kevzara**
- Solymbic
- **Spherox**
- Tacforius
- **Tremyfa**
- **Xeljanz**

Neurology

- **Brineura**
- Lacosamide Accord
- Mavenclad
- Nyxoid
- **Ocrevus**
- **Oxervate**
- **Reagila**
- **Spinraza**
- Verkazia
- Zubsolv

Uro-nephrology

- Elmiron
- **Lokelma**
- Tadalafil Lilly
- **Veltassa**

Haematology/ Haemostaseology

- **Adynovi**
- Anagrelide Mylan
- **Refixia**
- VeraSeal

Pneumology/ Allergology

- Elebrato Ellipta
- **Fasenra**
- Rolufta
- Trelegy Ellipta
- Trimbow

Dermatology

- **Dupixent**
- **Kyntheum**
- Skilarence

Hepatology/ Gastroenterology

- Jorveza
- **Alofisel**

Metabolism

- Cuprior
- Febuxostat Mylan

Cardiovascular

- Roteas

2017- indikace nově registrovaných „orphanů“

- Merkel cell carcinoma
- Precursor Cell Lymphoblastic Leukemia-Lymphoma
- Neuronal ceroid lipofuscinosis type 2
- Cystinosis
- Neuroblastoma
- Mycosis fungoides-type cutaneous,T-cell lymphoma
- Hypoparathyroidism
- Neurotrophic keratitis
- Acute myeloid leukemia, systemic mastocytosis
- Spinal muscular atrophy
- Carcinoid syndrome
- Fallopian tube neoplasms, ovarian neoplasms, peritoneal neoplasms

Současná situace v ČR

- **2 roky! trvá přechod léku do režimu trvalé úhrady**
- §16 - jediná možná léčba
  - **můžeme léčit jako před 30 lety**
- Rozdílný a nepředvídatelný přístup plátců k úhradě
- Zlepšující se ekonomická situace umožňuje přístup k nejefektivnější léčbě

Děkuji za pozornost

Děkuji za budoucí spolupráci

Jménem pacientů
Jménem budoucích pacientů, kteří o tom ještě nevědí
Jménem příbuzných a přátel pacientů současných i budoucích
Jménem těch, kteří budou mít i díky vám šanci žít
Jménem těch, kteří už tu možnost nemají