



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Press office

Recommendations on eligibility to PRIME scheme

Adopted at the CHMP meeting of 9-12 December 2019

During its December 2019 meeting, the CHMP reviewed 6 recommendations for eligibility to PRIME: 2 were granted and 4 were denied. The individual outcomes adopted this month are listed below.

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Eligibility granted

Name*	Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
Autologous CD34+ enriched cells transduced ex vivo with lentiviral vector carrying the FANCA gene, PGK-FANCA-WPRE (RP-L102)	Advanced Therapy	Haematology-haemostaseology	Treatment of Fanconi anaemia Type A	Nonclinical+ Clinical exploratory	Other
Rebisufligene etisparvovec	Advanced Therapy	Endocrinology-Gynaecology-Fertility-Metabolism	Treatment of Mucopolysaccharidosis Type IIIA, MPS IIIA (Sanfilippo A Syndrome)	Nonclinical+ Clinical exploratory	SME

* Name of the active substance, INN, common name, chemical name or company code.

SME applicants are micro-, small-and medium-sized-enterprises registered with the Agency's SME office. Other types of applicants are those not qualifying or not registered as SME or not fulfilling the definition of academic sponsors.

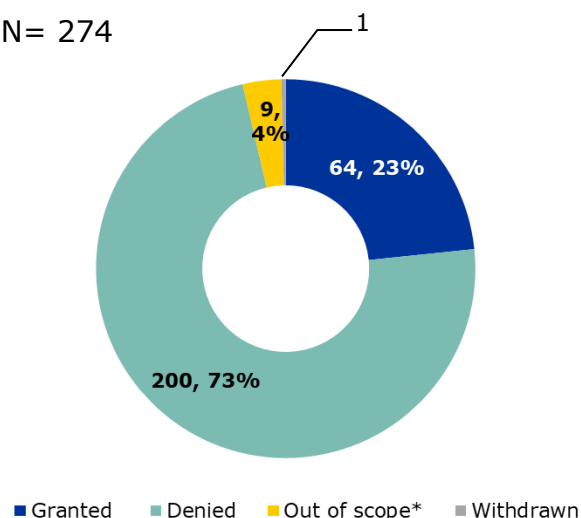
Eligibility denied

Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
Biological	Oncology	Treatment of recurrent glioblastoma	Nonclinical+ Clinical exploratory	Other
Chemical	Oncology	Treatment of patients with low or intermediate grade (G1 or G2) progressive, locally advanced or metastatic extra pancreatic NETs, which have been treated with at least 1 line of prior therapy	Nonclinical+ Clinical exploratory	Other
Biological	Endocrinology-Gynaecology-Fertility-Metabolism	Treatment of patients with Arginase 1 Deficiency (hyperargininaemia)	Nonclinical+ Clinical exploratory	SME
Chemical	Neurology	Treatment of mild to moderate Alzheimer's Disease	Nonclinical+ Clinical exploratory	Other

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Cumulative overview of recommendations on PRIME eligibility requests adopted by 12 December 2019

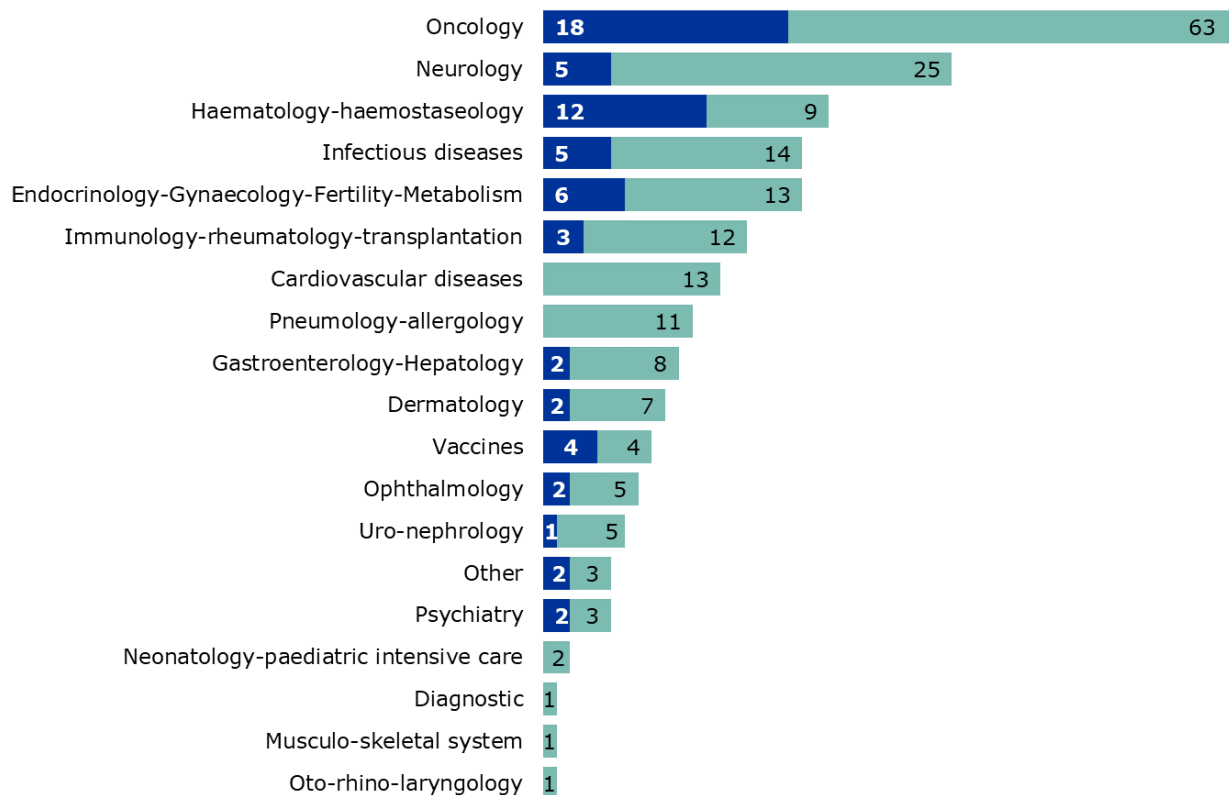
N= 274



By type of applicant



By therapeutic area



■ Granted ■ Denied

* This indicates eligibility requests received but not started by EMA as they were deemed outside the scope of the scheme or with a format and content inadequate to support their review. These are not included in the breakdown by type of applicant or by therapeutic area.