



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

20 November 2019  
EMA/506363/2019  
Press office

## Recommendations on eligibility to PRIME scheme

Adopted at the CHMP meeting of 11-14 November 2019

During its November 2019 meeting, the CHMP reviewed 8 recommendations for eligibility to PRIME: 2 were granted and 6 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 CD4+ and CD8+ T-cell populations transduced with a genetically-engineered replication-incompetent, self-inactivating lentiviral vector to express a BCMA-specific CAR (JCAR125)	Advanced Therapy	Oncology	Treatment of relapsed / refractory multiple myeloma whose prior therapies included autologous stem cell transplant if they were eligible, a proteasome inhibitor, an immunomodulatory agent and an anti-CD38 antibody	Nonclinical+ Clinical exploratory	Other
Danicopan	Chemical	Haematology - Hemostaseology	Treatment of paroxysmal nocturnal hemoglobinuria not adequately responding to a C5 inhibitor	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.

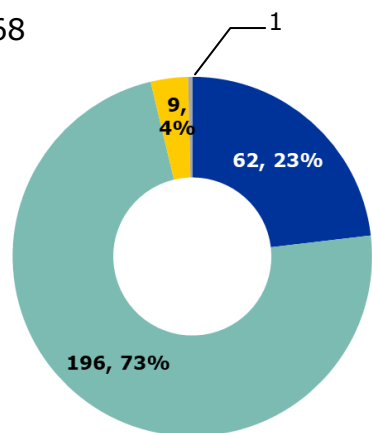
## Eligibility denied

Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
Chemical	Endocrinology-Gynaecology-Fertility-Metabolism	Treatment of postbariatric hypoglycemia	Nonclinical+ Clinical exploratory	SME
Chemical	Endocrinology-Gynaecology-Fertility-Metabolism	Treatment of inherited disorders of oxidative phosphorylation	Nonclinical+ Clinical exploratory	SME
Biological	Infectious diseases	Treatment of chronic Hepatitis D Virus Infection	Nonclinical+ Clinical exploratory	SME
Chemical	Neurology	Treatment of amyotrophic lateral sclerosis	Nonclinical+ Clinical exploratory	Other
Chemical	Neurology	Treatment of children with Rett Syndrome	Nonclinical+ Clinical exploratory	SME
Chemical	Neurology	Treatment of early Alzheimer's disease	Nonclinical+ Clinical exploratory	SME

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

## Cumulative overview of recommendations on PRIME eligibility requests adopted by 14 November 2019

N= 268

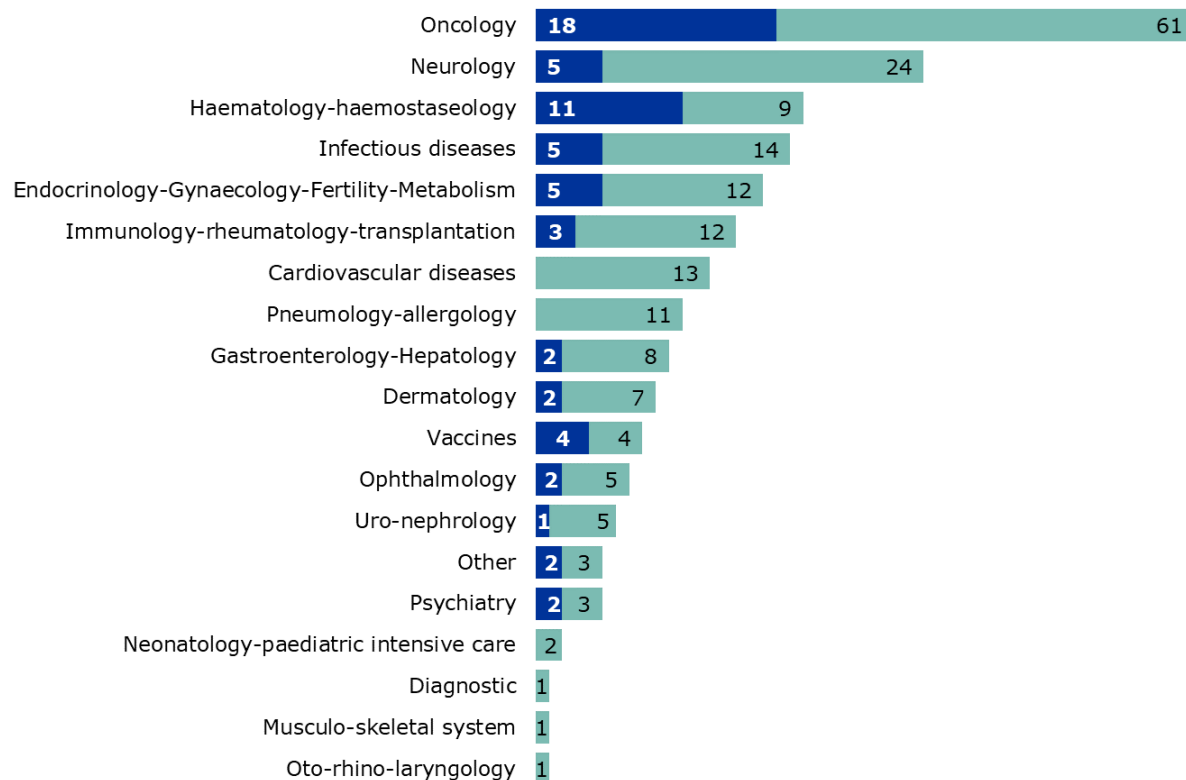


■ Granted ■ Denied ■ Out of scope\* ■ Withdrawn

### 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.