# Ready for prime time?

## *Have EMA prime designations supported accelerated regulatory approval and patient access?*

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## Background

- In March 2016, the EMA launched the PRIority MEdicines (PRIME) scheme aimed at enhancing early dialogue and regulatory support for putative medicinal products that address severe unmet treatment needs
- PRIME offers earlier application opportunities and scientific advice fee waivers to applicants from the academic sector and small and medium sized enterprises (SMEs)
- PRIME also enables EMA accelerated assessment (which reduces the assessment timeframe from 210 days to 150 days)
- This research aimed to systematically evaluate all PRIME designations to date and how these have translated into reimbursement and patient access

## Methods

- The EMA website was screened and medicines with PRIME designation identified (as of 06-MAR-2019). For any with EC marketing authorisation (MA), the corresponding HTA assessments by NICE, SMC, G-BA and HAS were screened and their outcomes extracted

## Results

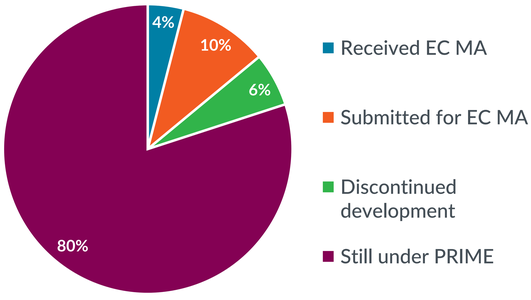
### *Number and type of therapies with PRIME designation*

- 50 therapies have received PRIME designations (2016: 9; 2017: 16; 2018: 13; 2019: 2)
  - The most common therapy indications were in oncology (26%) and haematology (18%)
  - 40% were submitted by SMEs

### *Status of drugs with PRIME designation*

- 20% no longer have PRIME designation (Figure 1)
  - 3 discontinued development,
  - 5 submitted for EC MA, and
  - 2 received EC MA: the CAR-T cell therapies: tisagenlecleucel [in ALL], and axicabtagene ciloleucel

*Figure 1: Proportion of PRIME designations by current status*




### *How have products approved under PRIME fared by EU HTA bodies?*

- All HTA bodies in scope had appraised both, 7/8 with positive outcomes (though 4/7 conditionally)
- The median delay from MA to positive HTA assessment was 4.7 months

*Table 1: HTA appraisals of currently marketed ATMPs*

| Brand name | INN | EC-approval date | NICE | SMC | G-BA | HAS |
|---|---|---|---|---|---|---|
| KYMRIAH® | Tisagenlecleucel | 22/08/2018 | 12/03/2019 (CDF) | 11/01/2019 (Accepted) | 17/12/2018 (Unquantifiable benefit*) | 12/12/2018 (ASMR III) |
| YESCARTA® | Axicabtagene ciloleucel | 23/08/2018 | 23/01/2019 (CDF) | 11/01/2019 (Not recommended) | 17/12/2018 (Unquantifiable benefit*) | 05/12/2018 (ASMR III) |

*Time-limited resolution: the G-BA requires Novartis to submit new data on Kymriah® for a new assessment in 2020

KEY: = positive decision = restricted/conditional decision = negative decision = no decision

## Conclusions

- Many therapies have received PRIME designation, but to date more have had their development discontinued than have received EC MA
- Nevertheless, the two EC-approved therapies with PRIME designations have received broad reimbursement across several major markets, but these recommendations are frequently conditional and add additional delays to patient access
- Better integration with payers may be necessary to ensure that accelerated regulatory approval programs translate into patent access

ALL: Acute lymphoblastic leukaemia; CAR-T: Chimeric antigen receptor T cell; CDF: Cancer Drugs Fund; EC: European Commission; EMA: European Medicines Agency; G-BA: Gemeinsamer Bundesausschuss; HAS: Haute Autorité de Santé; HTA: Health technology assessment; NICE: National Institute for Health and Care Excellence; MA: Marketing Authorisation; PRIME: PRIority MEdicines; SMC: Scottish Medicines Consortium



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