Does FDA Breakthrough Designation (BTD) affect HTA recommendation in terms of timing and outcome?

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Introduction

In an effort to expedite the approval of drugs treating serious illnesses or addressing unmet medical need, breakthrough therapy designation (BTD) has been used by the FDA. However, the question remains whether BTD translates into faster approval not only in the US but to other regulatory agencies, quicker decision from health technology assessments (HTA) and ultimately global availability of medicines.

Objectives

To compare the outcomes and timing from regulatory submission to jurisdictional HTA recommendation for new active substances (NASs) based on US BTD.

Methods

Data was collected on NASs approved by FDA in 2014-2016 (26 BTD NASs and 92 non-BTD NASs) to evaluate this question. These NASs were then tracked until the end of 2018 in terms of first HTA recommendations in Australia, Canada, France, Germany, Poland, Scotland and Sweden.

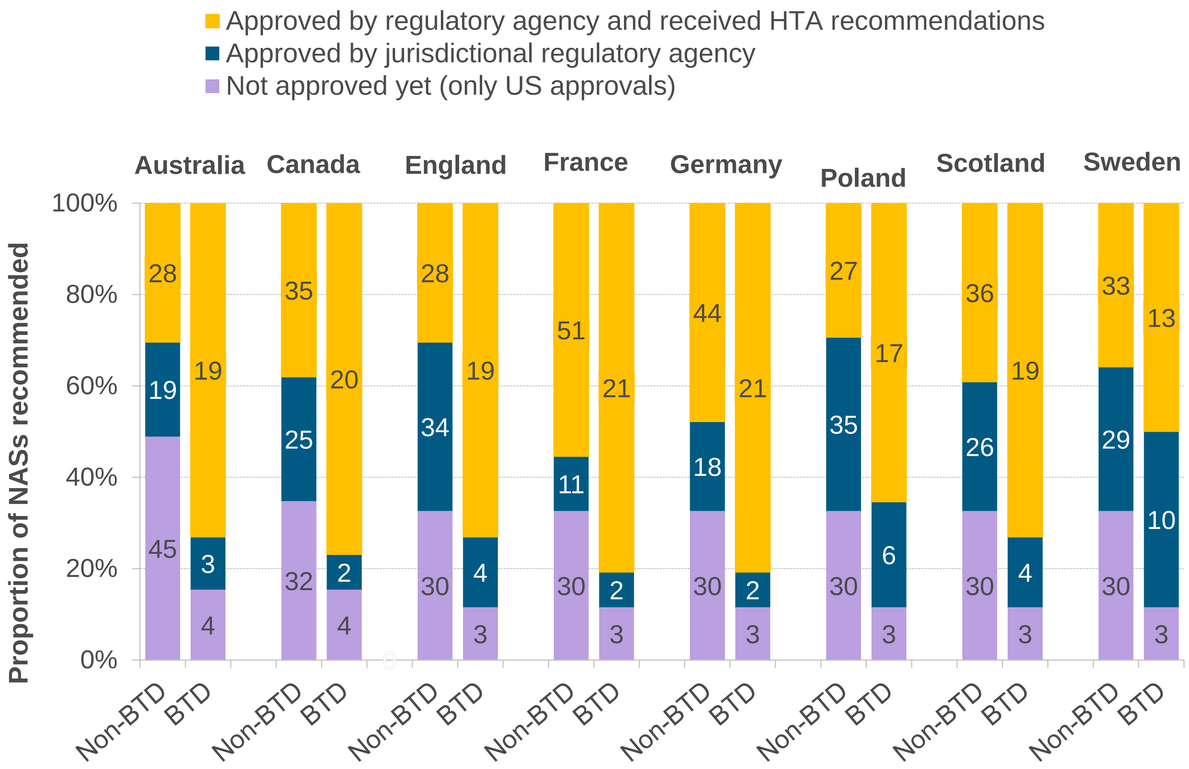
The data were then evaluated in terms of

- Timing (regulatory date of submission and approval, HTA recommendation date)
- HTA recommendation (positive, positive with restrictions, negative and multiple)

Effects of US BTD on HTA recommendation outcomes

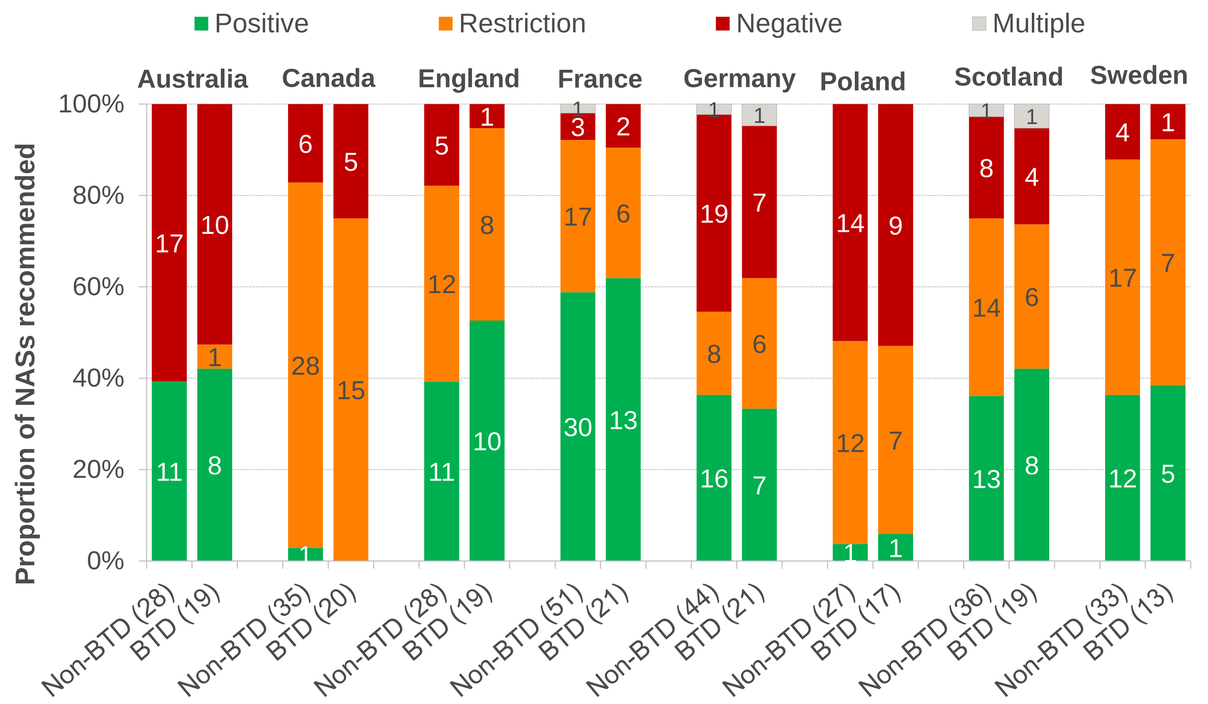
A higher proportion of BTD NASs reived a jurisdictional HTA recommendation as compared to non-BTD NASs (Fig 1).

Fig 1: Proportion of FDA-approved NAS that was approved by a jurisdictional regulatory agency and received a HTA recommendation



HTA recommendations were not affected by BTD status – BTDs do not lead to an increase in proportion of positive/positive with restrictions as compared to non-BTDs except in England, which has more than 10% difference and only 1 out of 13 common BTDs had all positive/ positive with restrictions recommendation across the jurisdictions (Fig 2).

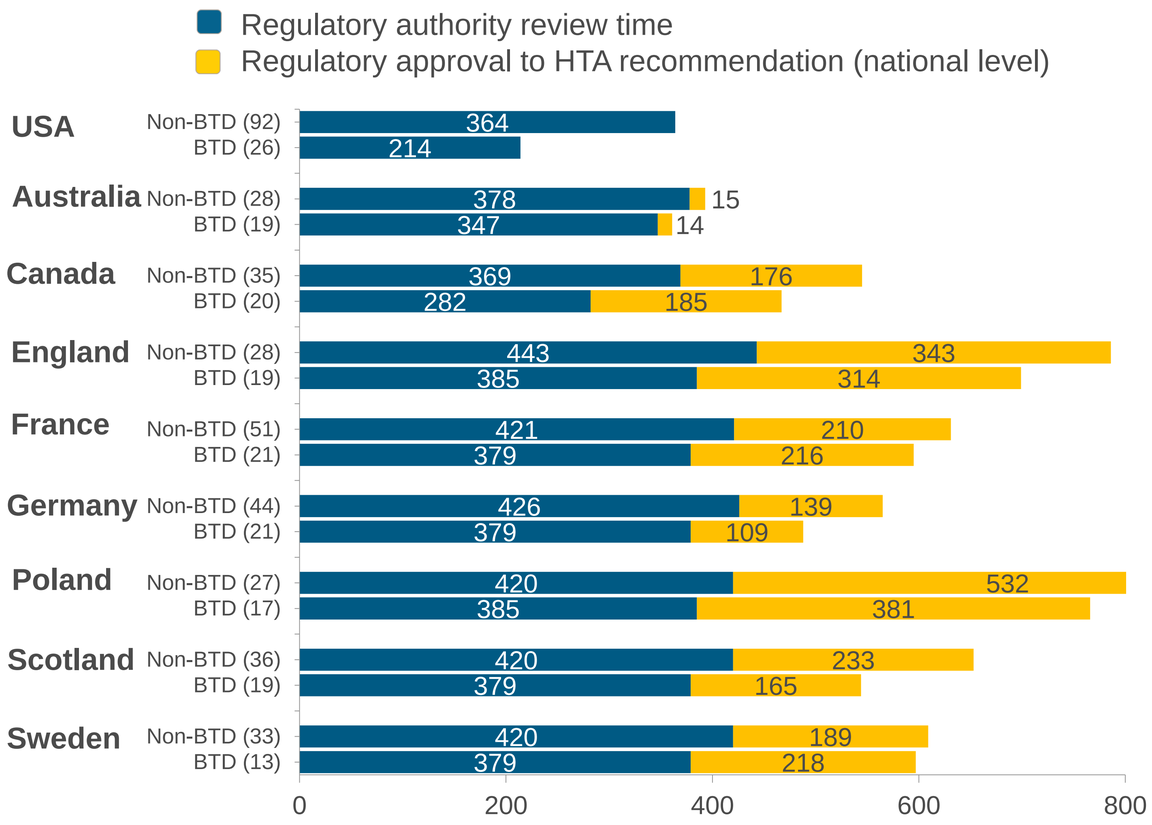
Fig 2: First HTA recommendation comparison by US BTD



Effects of US BTD on timing

In all the countries investigated, NASs with BTD had a faster overall time from regulatory submission to HTA recommendation in each jurisdiction as compared to non-BTD (Fig 3).

Fig 3: Breakdown of rollout time (days) by US BTD

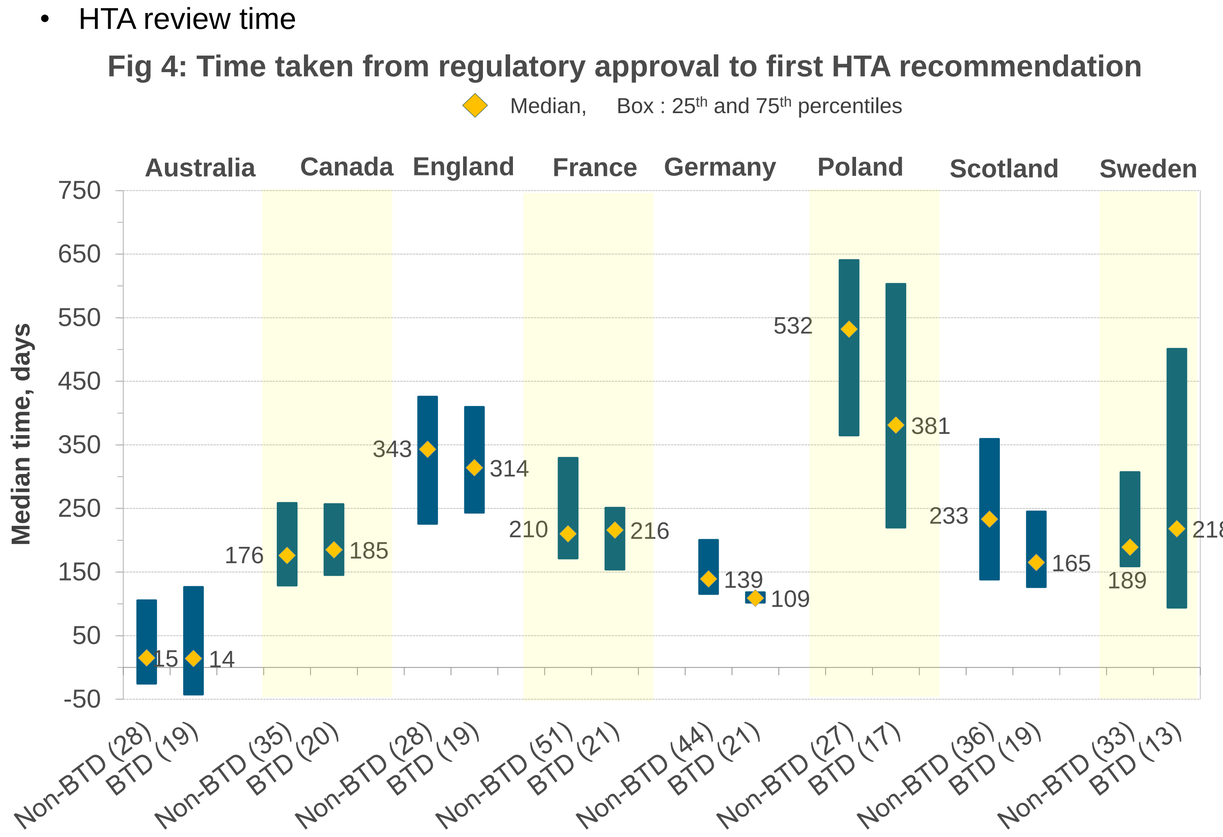


Interestingly, there was little time difference between regulatory approval and HTA recommendation at the jurisdictional level between BTDs and non-BTDs indicating that the reduction in overall time was driven by regulatory approval (Fig 4).

Time taken from regulatory approval to HTA recommendation includes:

- Company Strategy
- HTA review time

Fig 4: Time taken from regulatory approval to first HTA recommendation

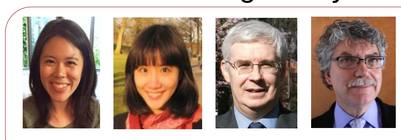


Conclusions

- These results suggest that getting a BTD designation does result to faster approval by other regulatory agencies. However, it does not translate to an increase in positive HTA outcomes.
- A better alignment between regulatory and HTA decisions could improve patient access to new drugs and presumably better predictability and less uncertainty.

Bibliography

Wang T, Cai J, McAuslane N, Munro J. 2019. R&D Briefing 73: Review of HTA outcomes and timelines in Australia, Canada and Europe 2014 -2018. Centre for Innovation in Regulatory Science. London, UK.



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Mission: To maintain a leadership role in identifying and applying scientific principles for the purpose of advancing regulatory and HTA policies and processes in developing and facilitating access to medicinal products.

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