

Reimbursement of New Orphan Medicines in Finland: A Survival Analysis of Access Delays



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Objectives

The availability of orphan medicines is an internationally recognized problem. Limited clinical evidence due to small patient populations has been proposed as a key contributing factor. The aim of this study was to examine whether outpatient orphan medicines reach reimbursement more slowly than other outpatient medicines in Finland, and whether differences are explained by their clinical added value or type of marketing authorisation.

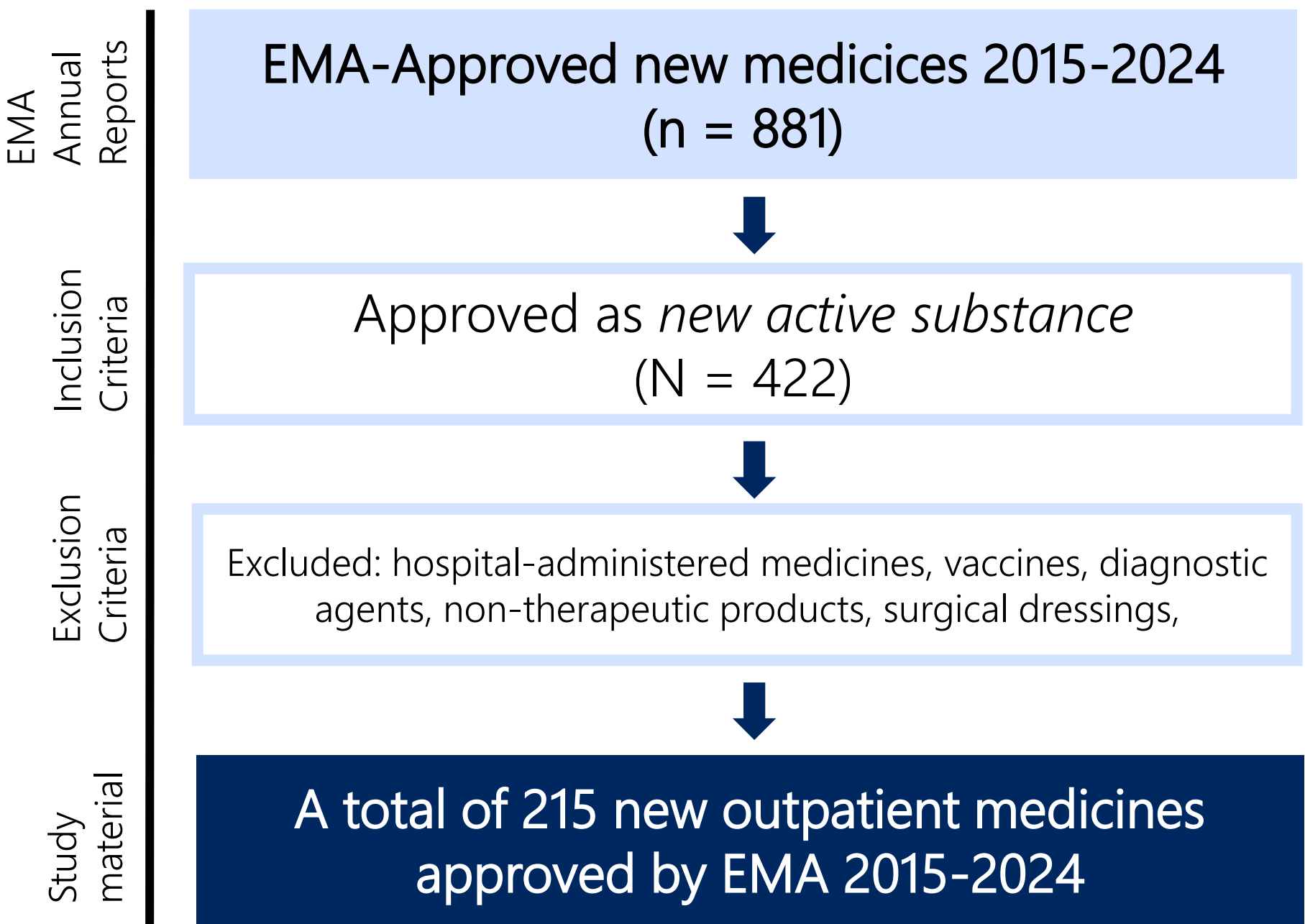
Materials and Methods

Outpatient medicines with a new active substance approved by European Medicines Agency (EMA) between 2015-2024 were included. Data on marketing authorisations were collected from EMA’s annual reports. Data on reimbursement status were collected from the notices of the Pharmaceuticals Pricing Board. Due to the confidential nature of Finnish assessments, Haute Autorité de Santé (HAS) evaluations were used as a proxy for clinical added value. The Kaplan-Meier method and the Cox proportional hazards model were used to estimate the association between orphan status and time to reimbursement, adjusting for clinical added value, authorisation year, and the type of marketing authorisation. The data cut-off date was 1 November 2025.

Results

A total of 215 outpatient medicines were included, of which 88 were orphan medicines (**Fig. 1**). Of the orphan medicines, 43 (49%) attained reimbursement, compared with 90 (71%) of non-orphan medicines. Orphan medicines reached reimbursement significantly more slowly (median 1100 [95% CI 883-2559] vs. 531 [412-655] days; log-rank test, $p = 0.004$) (**Fig. 2**). In the Cox regression analysis, orphan status was associated with a lower rate of reaching reimbursement. The association remained significant after adjustment for clinical added value, authorisation year, and authorisation type. (adjusted HR 0.30 [95% CI 0.18-0.52]; $p < 0.001$) (**Table 1**).

Figure 1: Data selection



Conclusions

Even after controlling for clinical added value and regulatory pathways, orphan designation predicts substantially delayed reimbursement. These findings suggest that factors beyond clinical uncertainty may contribute to delayed access to orphan medicines.

How are new outpatient medicines assessed in Finland?

In Finland, hospital medicines and outpatient medicines are assessed in separate processes. Pharmaceutical companies must apply to the Pharmaceuticals Pricing Board (PPB) if they wish to have an outpatient medicine included in the national reimbursement system. The assessment process may take no longer than 180 days.

Because reimbursement is application-based, access delays may result not only from the PPB’s application processing time, but also from the time between marketing authorisation and submission of a reimbursement application.

Figure 2: Kaplan-Meier analysis of time to reimbursement

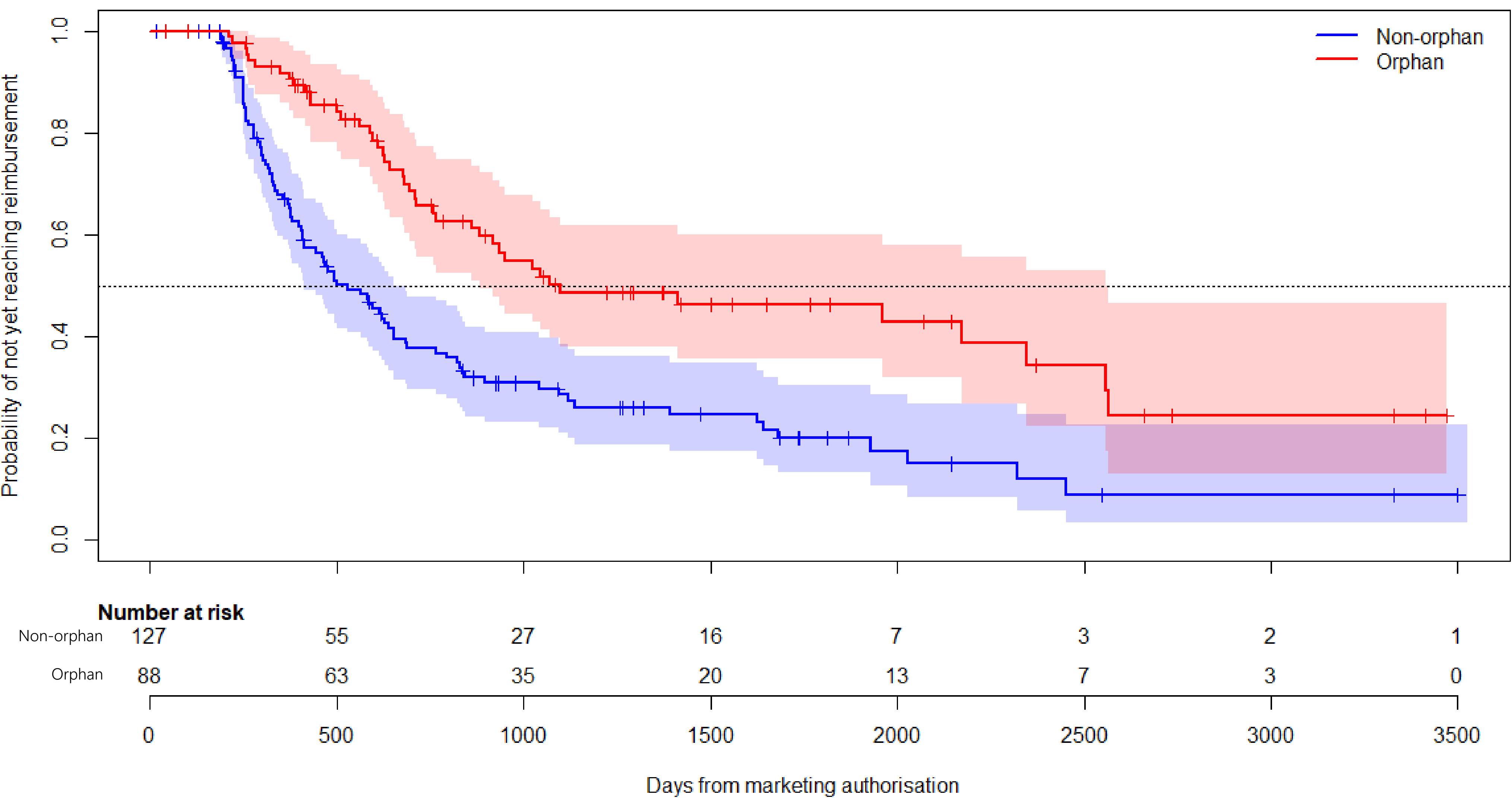


Table 1: Cox proportional hazards model

Variable	Comparison	Adjusted HR [95% CI]
Orphan status	Orphan vs Non-Orphan	0.30 [0.18-0.52]
HAS assessment (clinical added value)	HAS 1–3 vs. HAS 4–5/Insufficient	1.27 [0.71-2.28]
Authorisation year	Per 1-year increase	0.90 [0.79-1.03]
Authorisation type	Conditional/Exceptional vs. Standard	0.83 [0.40-1.71]

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