The value of Rare Pediatric Disease Designation program for decision making by drug developers

HPR216

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OBJECTIVES

Only an estimated 5 percent of rare pediatric diseases have a treatment, although more than ten million children in the US are affected. The Rare Pediatric Disease (RPD) Priority Review Voucher (PRV) program was established in 2012 to incentivize biotech company to develop rare pediatric disease drugs. This study investigates the potential of the program to promote a decision making for biotech company to move forward their rare pediatric disease drug development, by analyzing investor responses to the RPD designation announcement.

METHOD

We used the event study to analyze the magnitude of the investor's response to the announcement that biotech company has been awarded a RPD designation during 2020-2024. We choose this study period because the vast majority of RPD designation has been awarded after 2020. The statistical significance of the cumulative abnormal returns (CARs) is assessed by a non-parametric generalized rank t-test (GRANK-T test) with the significance level set at 5%. Statistical analyses were performed using EZR (version 4.4.2).

RESULTS

A total of 197 announcements were found in the study period. Following the announcement of a RPD designation, a company's stock price statistically significant increased on average by 5.54% (overall CARs) compared to what it would have been without the announcement (p=0.0094).

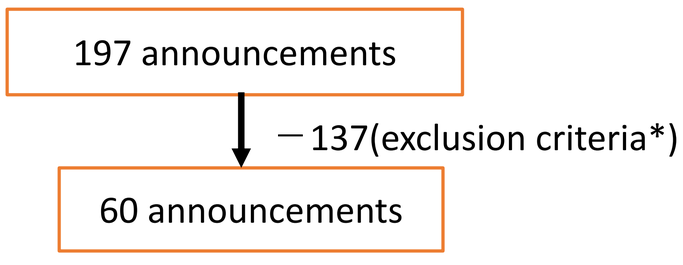

Figure 1. Data analysis and sample sizes

Table 1. Results of analysis

Sample	CARs	GRANK-T Test (p-value)
n=60	5.54%	2.64 (0.0094)

*exclusion criteria: 1. Not exist stock price data, 2.Confounding corporate-level event occurs

Numerous studies have used the event study methodology to determine how public announcements are used by pharmaceutical firms as signals to their investors. Miller *et al.* (2016 & 2017) reported a summary of recent event study results, and a comparison of these studies is shown in Table 2 as a reference.

Table 2. Comparison of Pharmaceutical Event Studies

Article	Central event	Study period	Sample	Return
Dedman *et al.* (2008)	Successful P3 trial results	1990-1998	N=27	1.56%
Sharma and Lacey(2004)	Drug approval	All confirmed FDA approvals	N=334	1.36%
Sarkar and de Jong(2006)	Drug approval	1990-2001	N=49	0.35%
Sturm *et al.* (2007)	Drug approval	1985-2004	N=196	1.53%
Ahmed *et al.* (2002)	Drug withdrawal	1966-1998	N=70	7.85%
Alefantis *et al.* (2004)	Fast-track designation	1998-2001	N=26	10.15%
Anderson and Zhang(2010)	Fast-track designation	1998-2004	N=109	10.11%
Miller *et al.* (2016)	Fast-track designation	1998-2015	N=238	6.25%
Miller *et al.* (2017)	Orphan drug designation	1985-2015	N=323	3.36%
Kazufumi *et al.* (this poster)	**Rare pediatric disease designation**	**2020-2024**	**N=60**	**5.54%**

DISCUSSION and CONCLUSIONS

These results suggest that the designation has been effective in this area: investors assign positive, statistically significant, value to the RPD designation. Positive stock market reactions to RPD designation announcements provides strong evidence that investors value a company's investment in the R&D of pediatric orphan drugs. Positive investor signals imply that this program is likely to promote a decision making for biotech company to move forward RPD drug development. However, our study period was limited to analyze the value of RPD designation which has been terminated by the end of 2024. We believe that further research is needed to evaluate this program and to report future development status of rare pediatric diseases.

REFERENCES

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