A Review of Patient-Reported Outcome (PRO) Label Claims in the United States From January 2006-September 2013: Analysis of Evidence for Orphan Drug PRO Label Claims

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ABSTRACT

Objective: Previous reviews of patient-reported outcome (PRO) label claims did not distinguish products with orphan designation. Literature suggests that less evidence is required for orphan drug approval than non-orphan drugs. This study aimed to analyze the frequency of measured characteristics for orphan and non-orphan drug approval claims. Methods: Using the Drug@FDA website, new orphan andOrphan product. PRO claims achieved for orphan products appear to have had priority review. We used the drug approval reports by month search on the Drugs@FDA website, focusing on orphan product classification. The patient population in the PRO validation study was not representative of the patients participating in the phase 3 study. VDS made this measure less reliable. Across trials, Firazyr median time to 50% reduction from baseline: 2.0-2.3 hours

As shown in Table 2, symptomatology and secondary endpoints achieved less than 25% of claims

No functioning-related claims were identified in our findings, whereas 25% of claims identified by Gnanasakthy and colleagues3 were for VAS items assessing skin swelling, skin pain, and abdominal discomfort. The patient population in the PRO validation study was not representative of the patients participating in the phase 3 study. VDS made this measure less reliable. Across trials, Firazyr median time to 50% reduction from baseline: 2.0-2.3 hours

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Priorities were unadministered

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