**RESULTS (CONTINUED)**

**Figure 3. Modules 3 Stratified by Number of Used Data Sources (N=103)**

- In 66 (64.0%) Modules 3, more than one data source was used with most modules relying on two data sources (Figure 3).

**Figure 4. Transparency of TP Size Determination by Number of Data Sources**

- In 71 (68.9%) Modules 3, the approach to determine the TP was deemed transparent according to the IQWiG assessment. In contrast, the determination of the TP was found to be non-transparent in 25 (24.3%) modules. For seven (6.8%) Modules 3, no information on transparency was available in the corresponding IQWiG assessments.

- Of those Modules 3 using only one data source to calculate the TP size, 24 (64.9%) were considered as transparent by the IQWiG. Moreover, 38 (77.6%) of the modules relying on two different data sources and five (38.5%) of the modules using three sources received a transparent assessment by the IQWiG. Beyond that, all identified Modules 3, which utilized more than three different data sources for the TP size determination were deemed transparent by the IQWiG (Figure 4).

**CONCLUSIONS**

- Most Modules 3 relied on two or more different data sources. For most of the modules, the IQWiG assessed the TP size determination to be transparent. However, only in 26.0% of all modules the TP size was deemed to be plausible.

- The results show that even if information on prevalence and incidence for rare diseases is available and a transparent approach is used, determining the TP size of a new orphan drug can be challenging.