THE DUCHENNE REGISTRY

The Duchenne Registry

The Duchenne Registry is an international, self-report registry and resource for individuals with Duchenne or Becker muscular dystrophy.

The Duchenne Registry: We've Got an App for That

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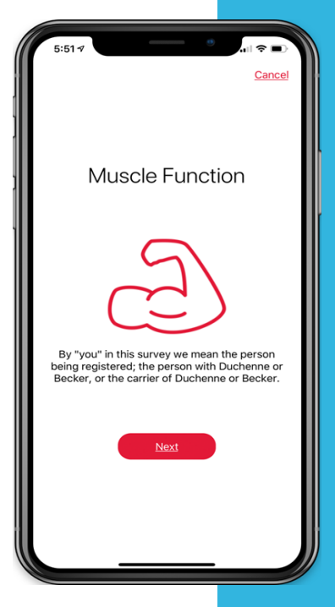
1. Parent Project Muscular Dystrophy

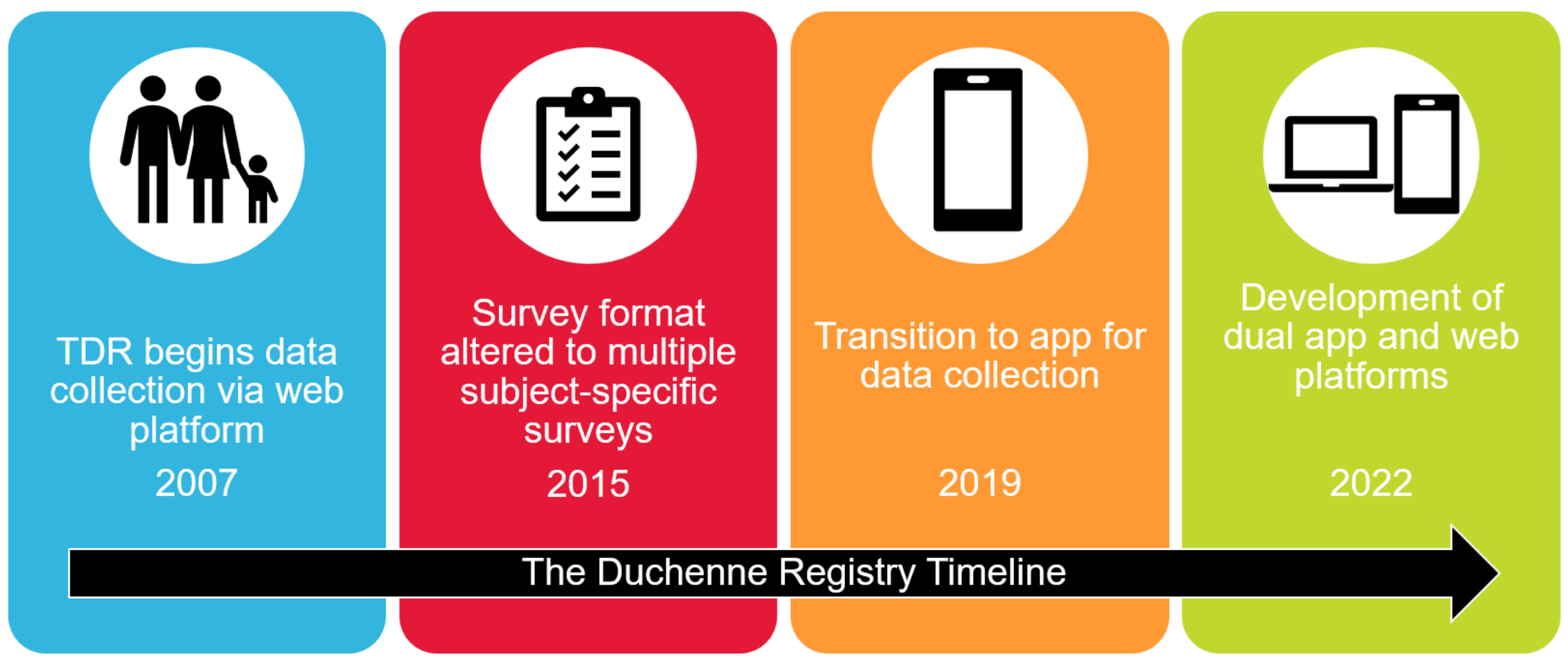


Muscle Function

By "you" in this survey we mean the person being registered; the person with Duchenne or Becker, or the carrier of Duchenne or Becker.

Background

The Duchenne Registry (TDR) is an international patient-report registry based in the USA that collects data on individuals with Duchenne or Becker muscular dystrophy as well as those who are carriers. TDR was initially deployed as a web-based platform in 2007. In 2019, TDR transitioned to an app-based platform in hopes of increasing ease of use for families and capitalizing on the popularity of apps; active participants complete surveys through the app on their smart phones or tablet. The app is currently collecting 15 surveys, covering the affected body systems, clinical trials, genetic testing, and pain. Since mid-2021, the app has enabled participants to submit photographs of their genetic test reports, which are then curated by genetic counselors.



TDR begins data collection via web platform
2007

Survey format altered to multiple subject-specific surveys
2015

Transition to app for data collection
2019

Development of dual app and web platforms
2022

The Duchenne Registry Timeline

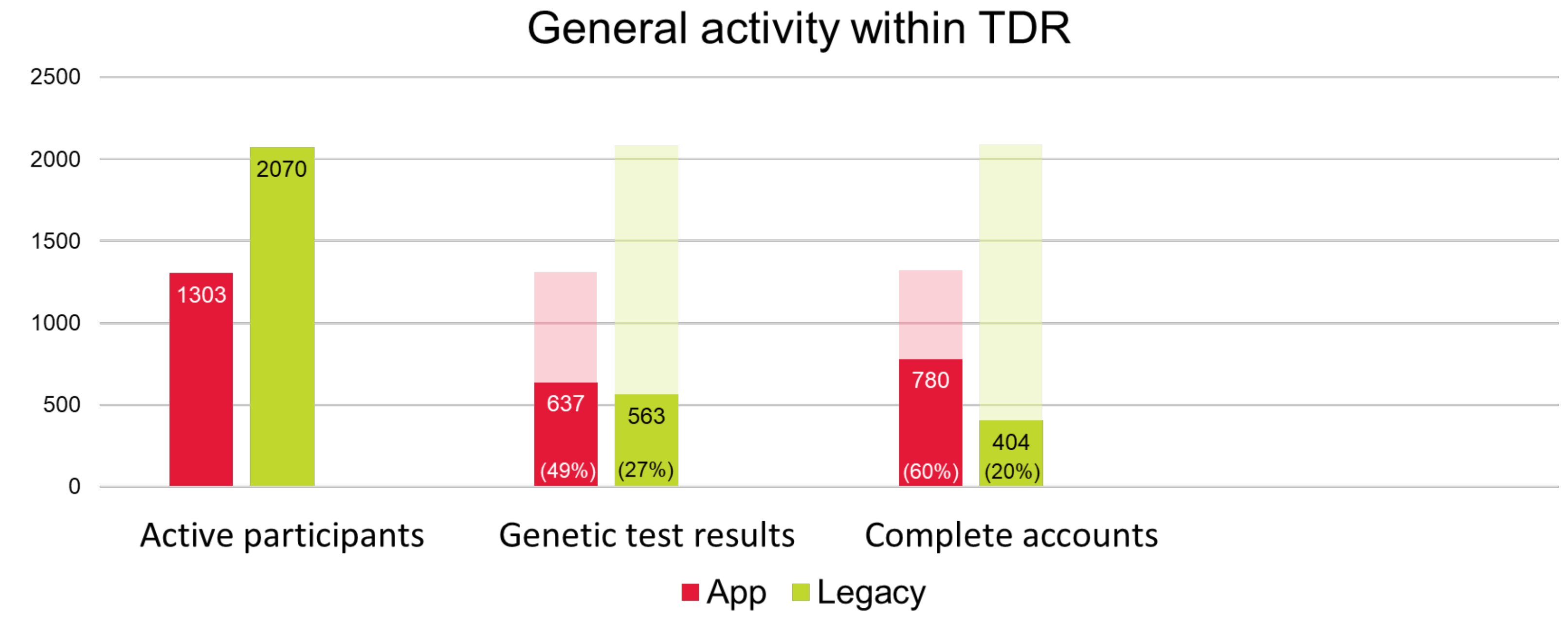
Methods

We compared data within TDR collected through the web portal (November 2016-August 2019, termed legacy data) immediately prior to the transition to the app, and data collected in the app (November 2019-August 2022, termed app data), immediately after the transition to the app, using an equivalent period of 34 months for each dataset. Data analysis was performed using the Prometheus Research platform.

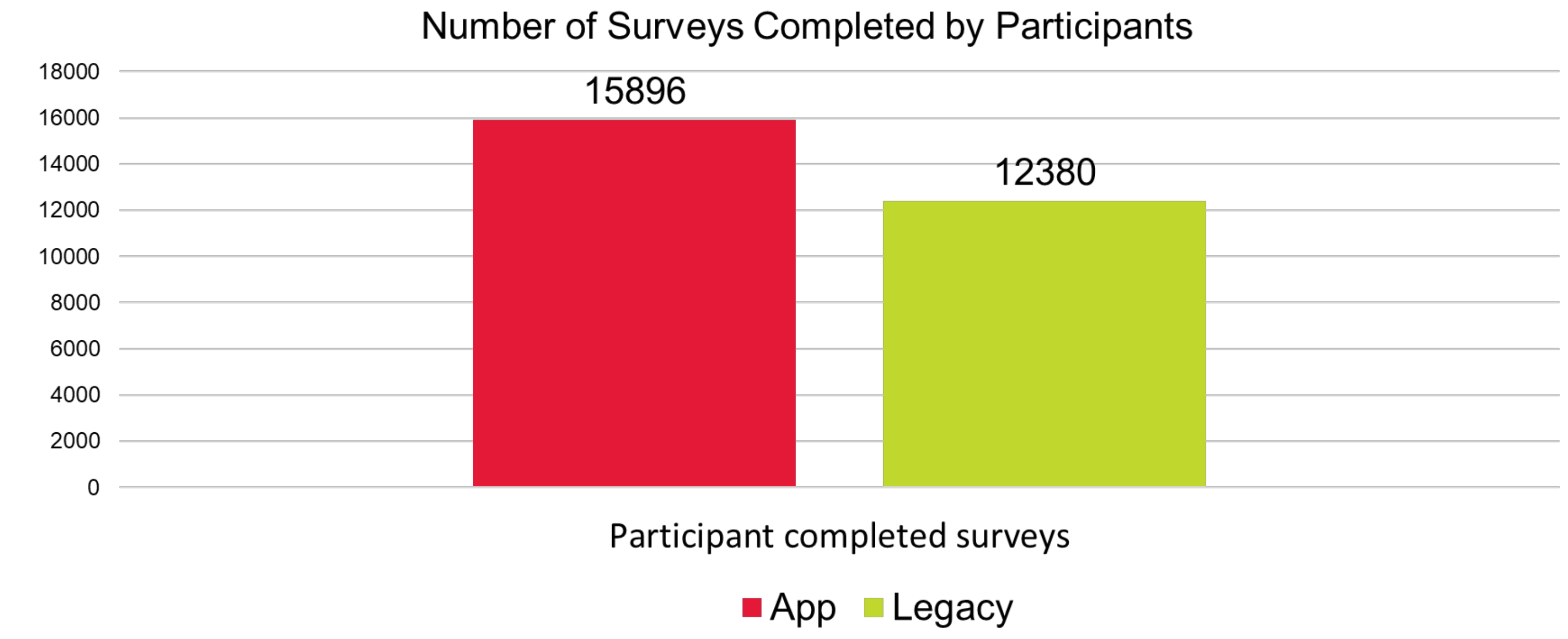
Results

General Activity

The app had 1303 active participants, versus 2079 participants active in the legacy dataset. More participants completed all eleven original surveys in the app, 60% having a complete data set, versus 20% of legacy participants.

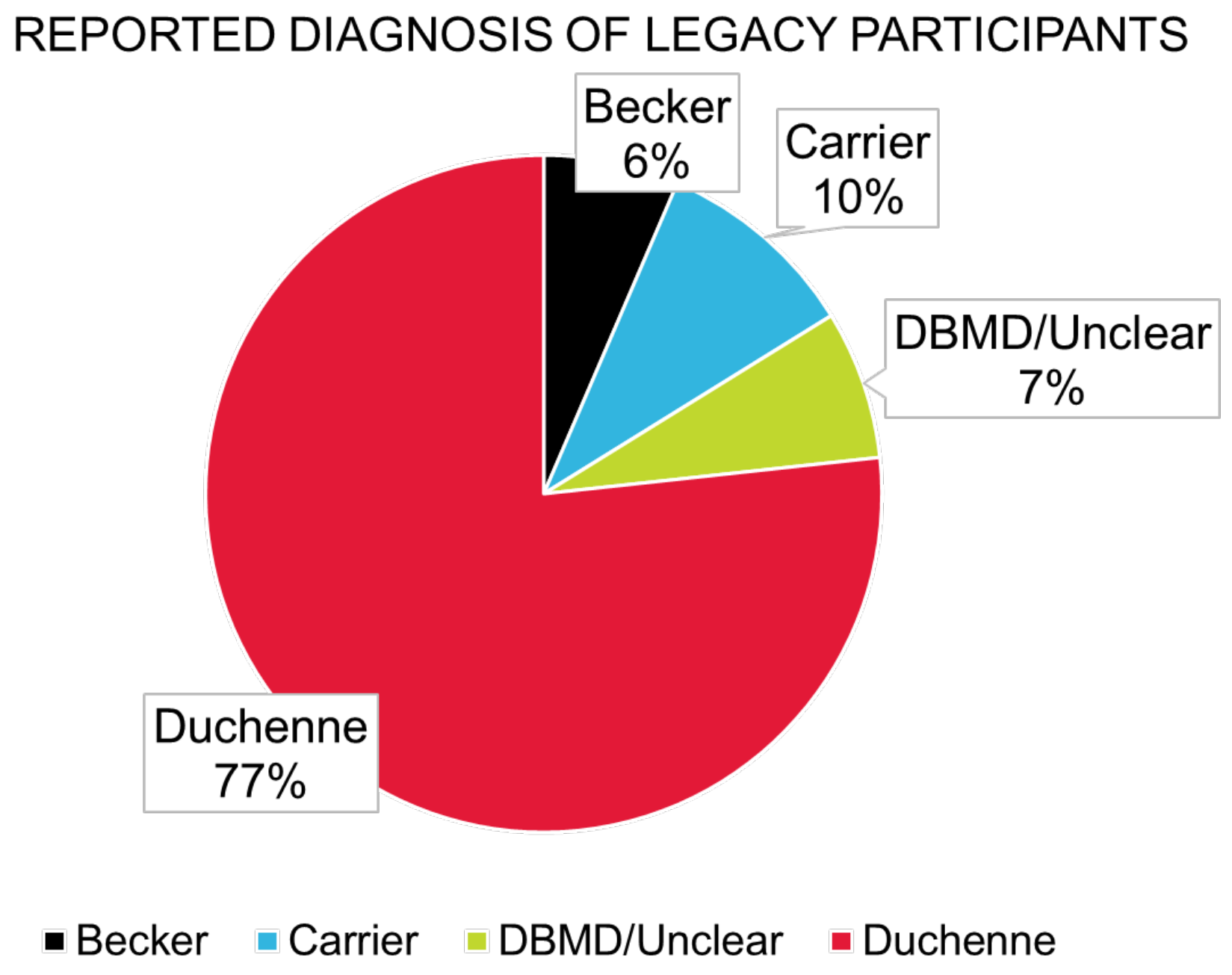


General activity within TDR

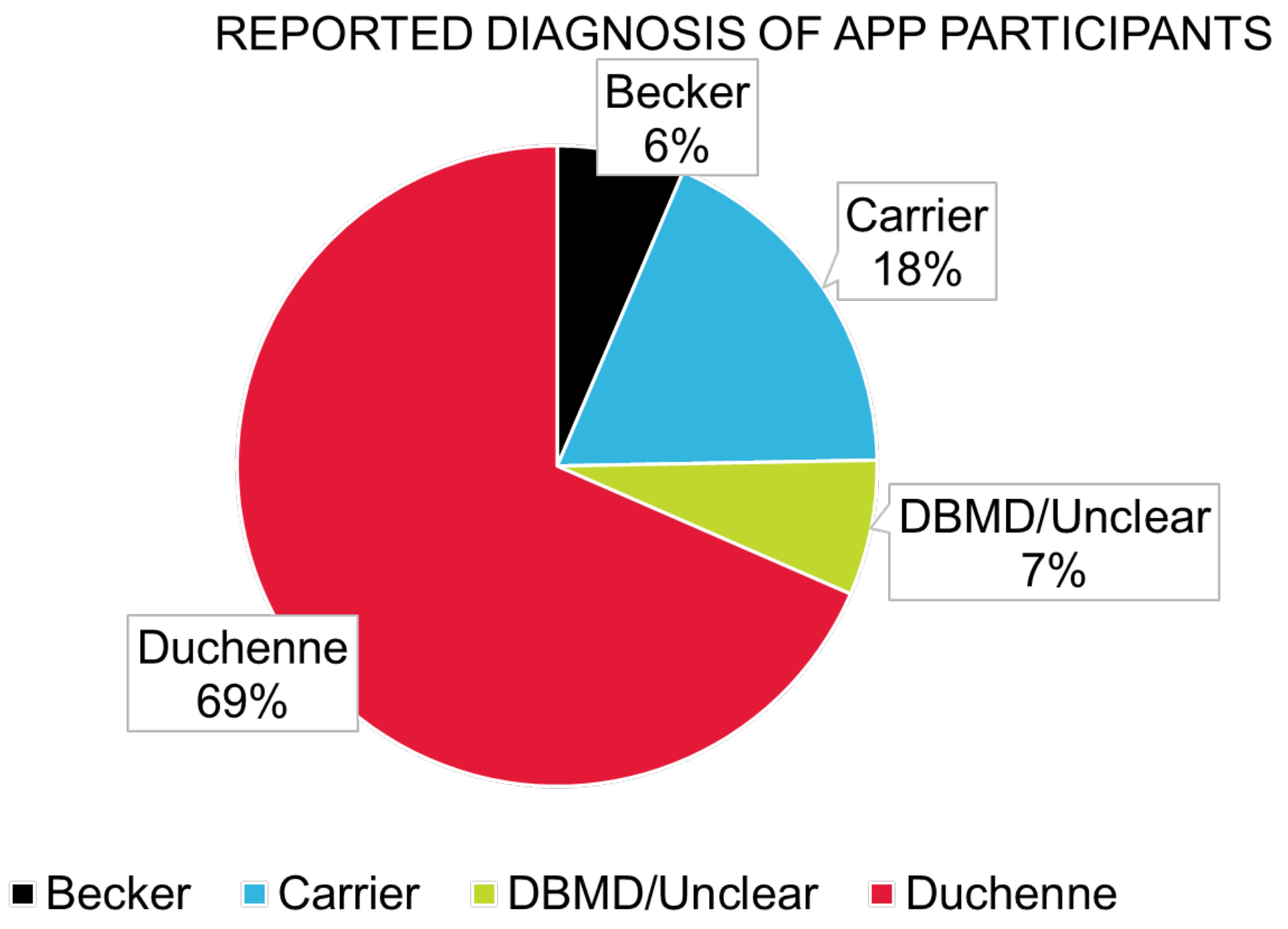


Number of Surveys Completed by Participants

Demographics



REPORTED DIAGNOSIS OF LEGACY PARTICIPANTS

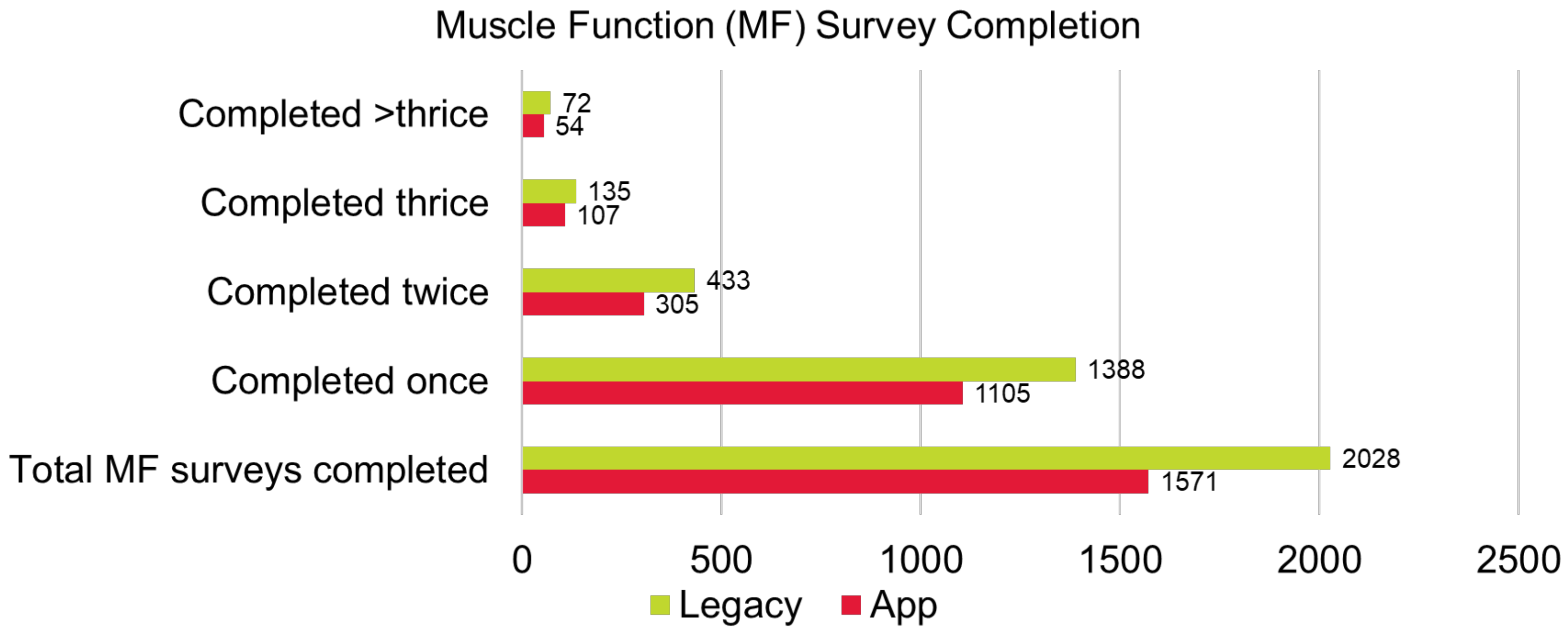


REPORTED DIAGNOSIS OF APP PARTICIPANTS

Duchenne is the most common diagnosis in both datasets (69% in the app versus 77% of legacy participants). In both datasets, the average age of individuals with Duchenne at the time of participation was 11 years. The app data has a higher percentage of carriers (18% versus 10%), which likely reflects efforts to increase carrier participation as well as increased identification of carriers through expanded carrier screening.

Muscle Function

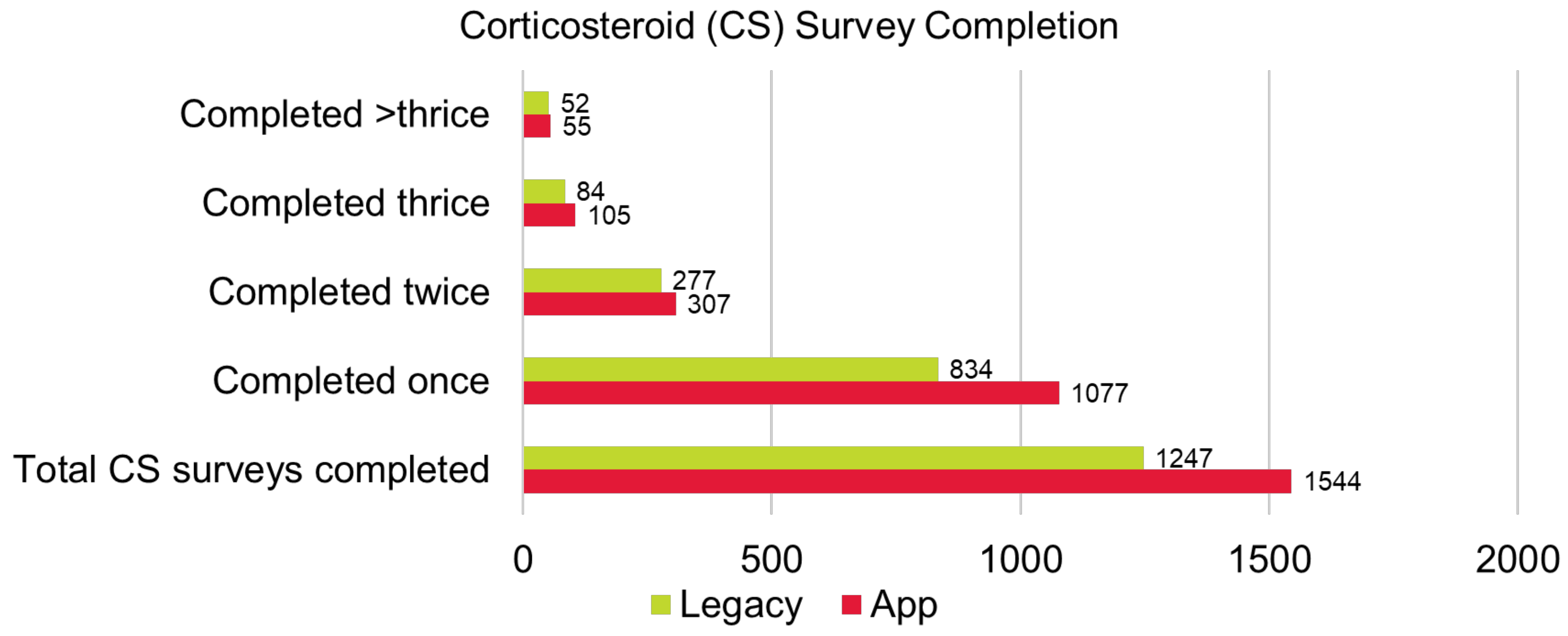
Muscle function (MF) is the most frequently completed survey within TDR in all datasets. It is also a very important survey, as it collects key data on ambulation and functional abilities. Participants are asked to complete the MF survey every six months, or at minimum, annually.



Muscle Function (MF) Survey Completion

Many participants in the legacy dataset only completed the demographics and the MF survey. Given that there are fewer overall participants in the app dataset, it is interesting that the number of participants who have completed the MF three or more times is relatively equivalent. Some participants who joined TDR toward the end of the time periods established for this study would not have been eligible to complete the survey more than once.

Corticosteroids



Corticosteroid (CS) Survey Completion

Corticosteroids (CS) is the next most frequently completed survey and collects data regarding corticosteroid use, type of steroid, dosing schedule, and age of initiation. CS was completed more frequently in the app, despite an overall lower number of participants. Interestingly, the number of participants completing CS thrice or more is very similar to the numbers completing MF thrice or more. On further review, a similar number have completed all the original eleven surveys three or more times, which suggests that there are a core group of participants who complete surveys on a regular, repeated basis.

Conclusions

TDR has always been adaptable, with content and number of surveys changing over time to correlate with TREAT-NMD data standards, participant interests, and broader research goals. Direct comparisons from the legacy data to the app are limited to specific elements and time periods. In this comparison, participants using the app were more likely to complete all surveys, resulting in a more thorough understanding of those participants, and were more likely to submit genetic test reports. Participant incentives in the app have often required all surveys be complete in order for participants to be eligible; it is possible that these incentives have driven survey completion. A similar number of participants completed the surveys more than three times, which is consistent for most surveys within TDR. However, there were fewer overall participants active in the app, potentially because of technology limitations or difficulties switching platforms.

Benefits of an app

- Convenience to families
- Accessibility
- Ability to push notifications

Potential pitfalls of an app

- Bugs may be device-specific and difficult to identify, given myriad of devices being used
- Requires users have the most recent version to access any updates

Because the two datasets occurred at different times, legacy collected pre-pandemic and app data primarily during and post-pandemic, it is difficult to know if decreased numbers of participants are related to fatigue with technology or other complicating social factors, or to differences in the method of data collection. Ultimately, having both a web and app platform may be ideal to give each participant the option of choosing a preferred platform. Consequently, TDR is currently developing a web portal option for families.

Acknowledgements

The app platform was developed by THREAD Research.
Thank you to all families and individuals who have participated in TDR!