

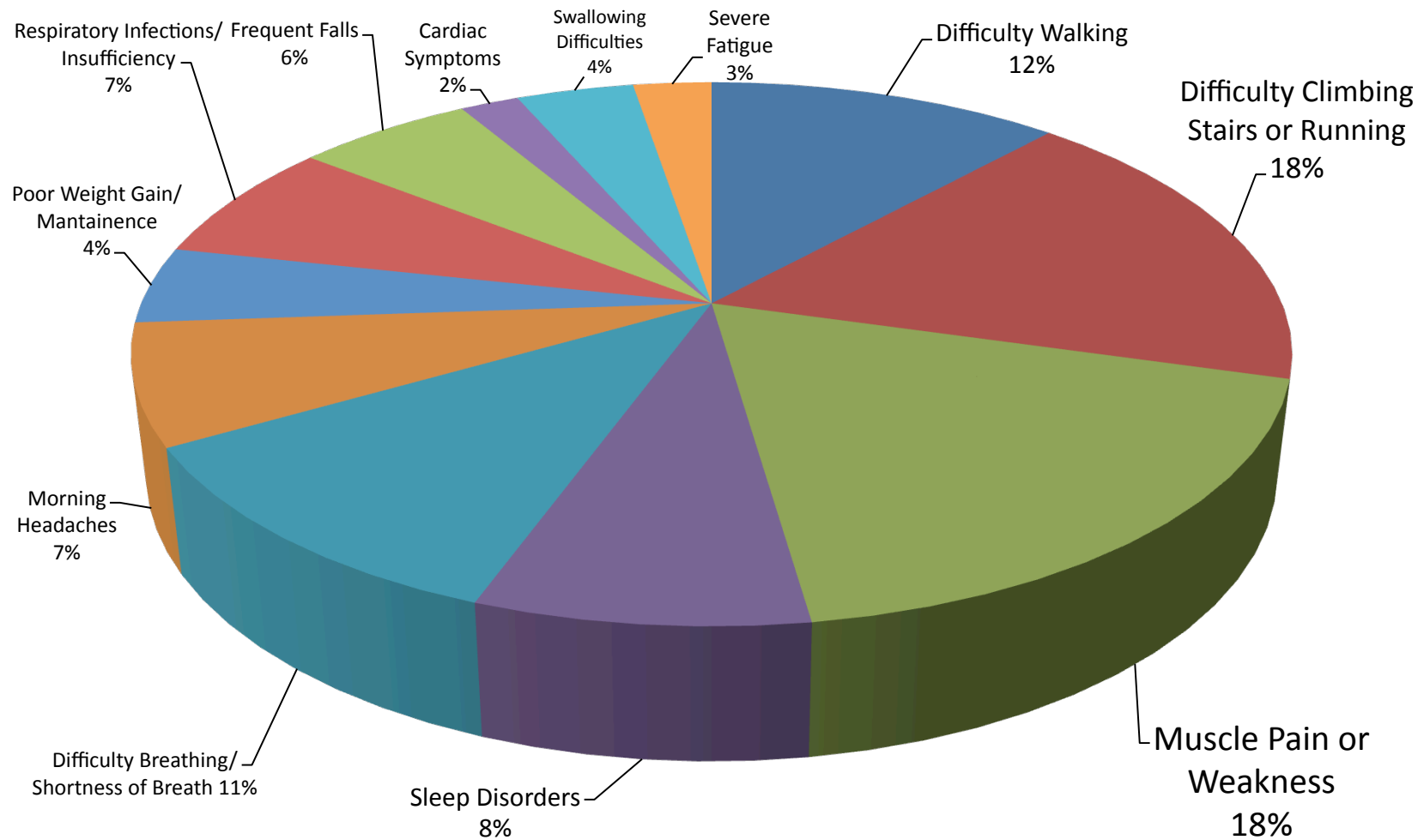
AMDA Path to Diagnosis Analysis

April 23, 2012

Background on Survey Data

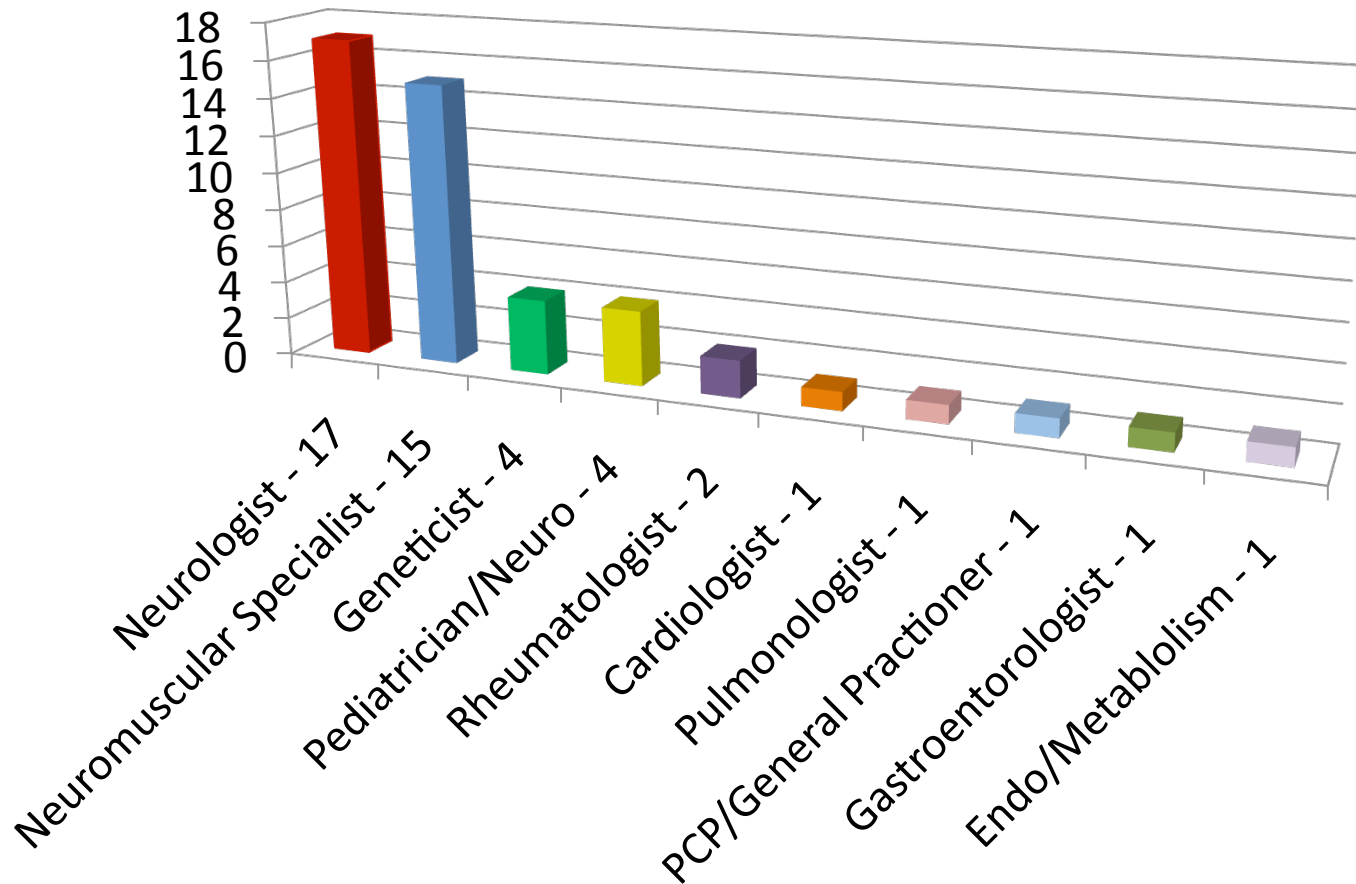
- Data in the following slides is based on the 49 AMDA surveys, which were submitted anonymously. As some questions may have been left blank, the following statistics are based on what data was actually provided.
- Of the 49 surveys that were provided:
 - 4 patients have infantile-onset Pompe
 - 36 have late-onset Pompe
 - 9 surveys did not provide adequate information to determine the phenotype
- 6 of the survey responses came from outside the U.S. The information collected from these surveys was included in each of the following statistics, as their responses were counted as representative of a Pompe patient's diagnosis journey regardless of citizenship. The external countries represented are Australia, South Africa, the Netherlands, the United Kingdom, France, and Canada
- Gender, age of patient and family history were not included in the survey and therefore do not influence the data.
- Some results were reported on behalf of a minor. Symptoms and answers listed reflect what the minor reported to the survey participant.

Indicate which symptoms you experienced that led you to seek medical attention.*



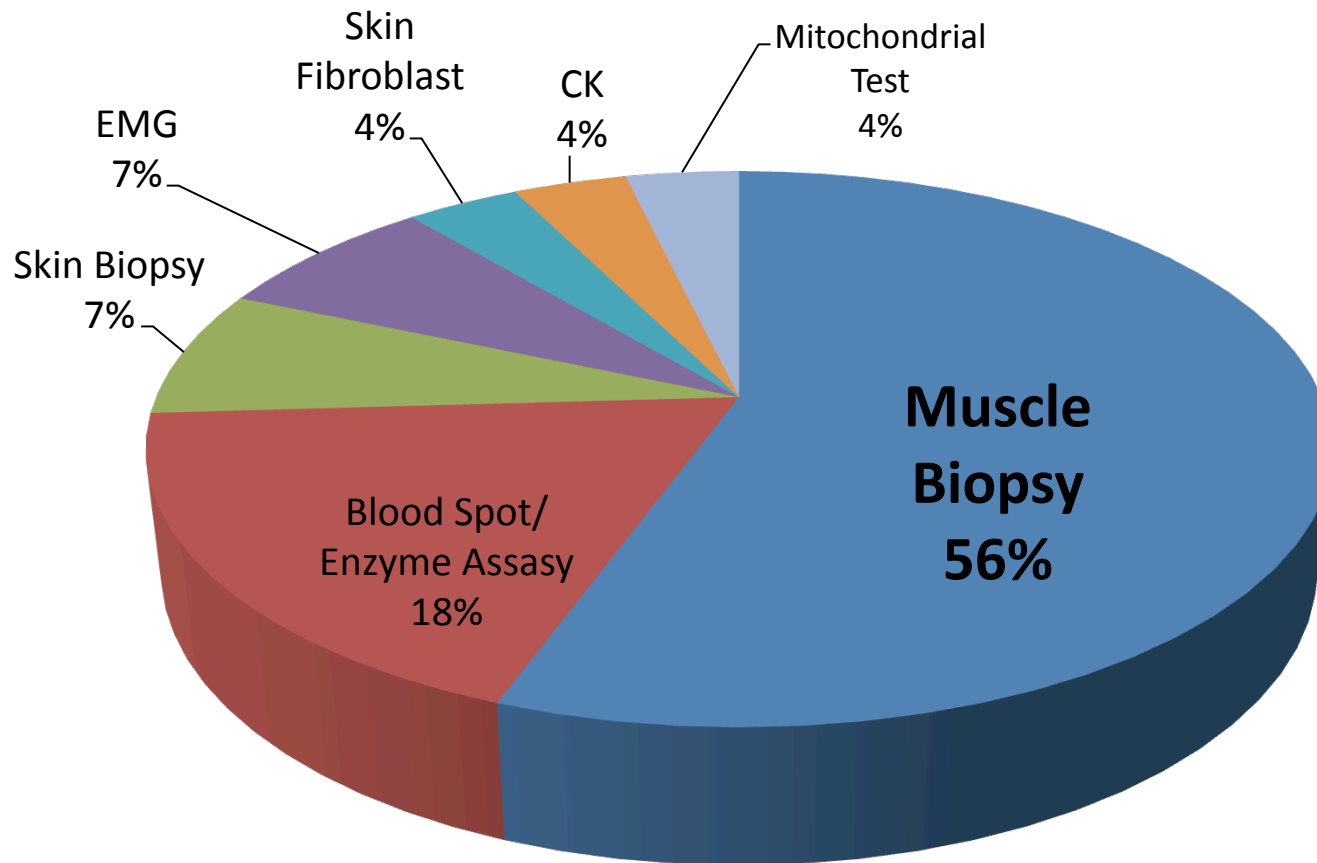
*Patients were asked to list all initial symptoms that first led them to see a doctor. Some participants noted that they did not recognize some of their experiences as symptoms until after receiving their Pompe diagnosis. The chart above reflects the frequency with which each symptom was listed by patients, regardless of when it occurred in their diagnosis process.

What physician specialty ultimately* diagnosed Pompe Disease?



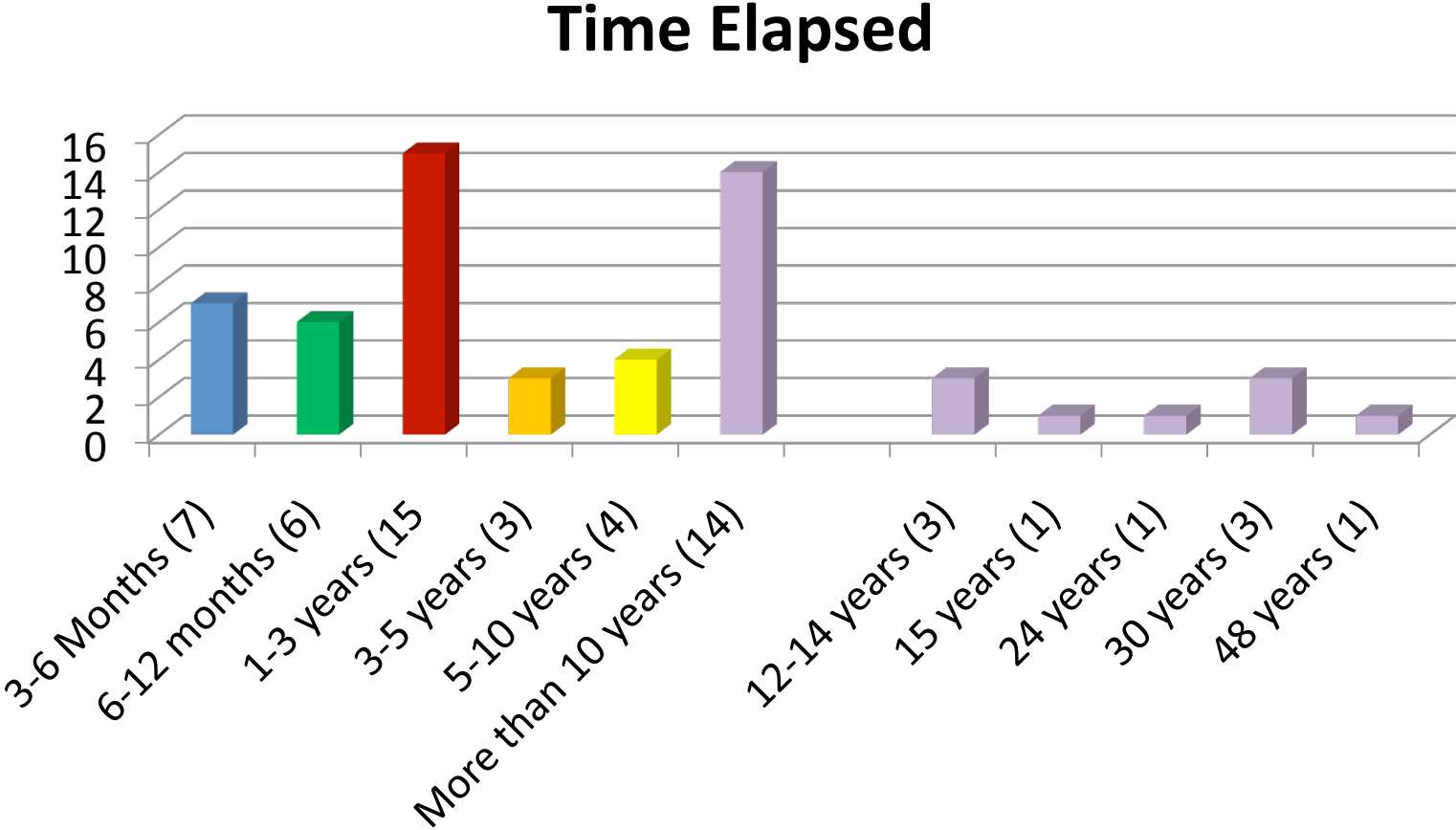
*Most patients visited several physicians during their diagnosis process. The chart above shows those physician who provided the official and final diagnosis of Pompe disease to survey participants. Other physicians the patient saw may have suggested Pompe disease or mentioned the disease to the patient without offering an official diagnosis.

What type of test* was used to reveal the Pompe diagnosis?

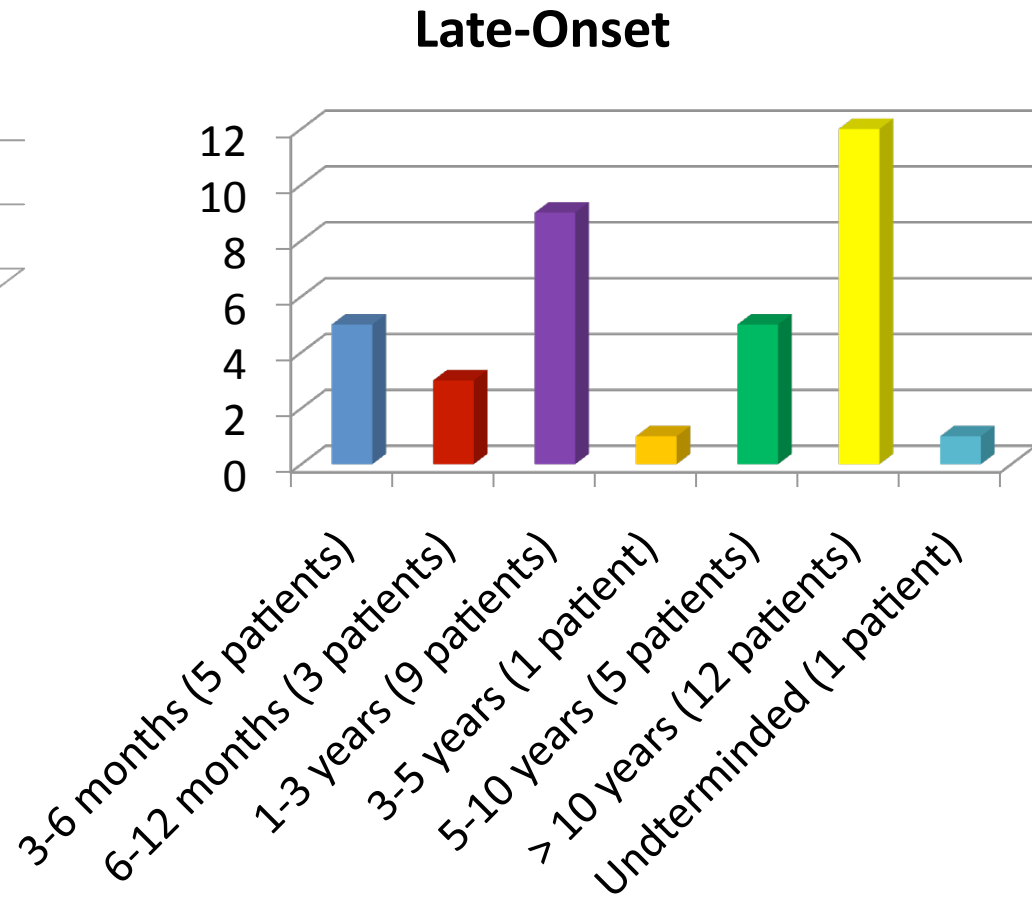
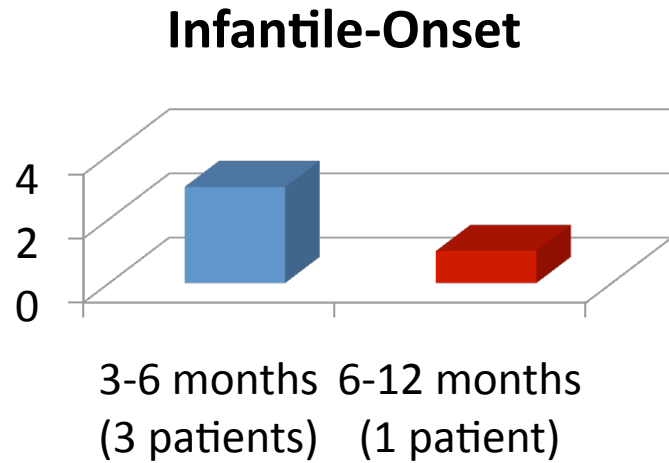


*This chart represents a breakdown of the tests listed that were used by a physician (though not necessarily the diagnosing physician) to lead to a Pompe diagnosis. Not all participants could recall which test was used that led to their Pompe diagnosis.

How much time elapsed from experiencing your initial symptoms until you received the diagnosis of Pompe Disease?



Time elapsed between symptoms and diagnosis: Infantile-Onset vs. Late-Onset*



*Of the 49 participant surveys received, it was determined that 4 patients have infantile-onset Pompe while 36 have late-onset. For 9 of the surveys the phenotype could not be determined and therefore that data is not included in the charts above.