genzyme
A SANOFI COMPANY

# ADVANCE Study Fact Sheet

### *What is the ADVANCE study?*

The ADVANCE study is an open-label prospective study in the United States evaluating the efficacy and safety of alglucosidase alfa produced at the 4000 L scale in Pompe patients 12 months and older previously treated with Myozyme® (alglucosidase alfa), which is produced at the 160L scale.

### *What is the purpose of the ADVANCE study?*

The study will evaluate the efficacy and safety of treatment with 4000 L scale alglucosidase alfa in patients 12 months of age and older with Pompe disease.

### *Who is eligible to enroll in the study?*

Patients may be eligible to participate if:

- They and/or their parent/legal guardian are willing and able to provide signed informed consent
- They have been diagnosed with Pompe disease and received treatment with 160 L scale alglucosidase alfa (Myozyme) prior to screening
- They, in the opinion of the Investigator, are considered to be medically stable and would be expected to complete the 52 week treatment period
- They are at least 1 year of age at time of consent
- They are not pregnant and will use medically accepted contraception throughout the study if they are of child-bearing age
- In the past 3 months (or currently), they have not received any investigational product and they are not currently participating in another clinical treatment study
- They are at least 1 year of age at time of consent

### *What treatment will patients receive while enrolled in the study?*

Patients will be treated with alglucosidase alfa produced at the 4000 L scale.

### *Is this a placebo-controlled study?*

No. All patients enrolled in the study will be treated with alglucosidase alfa produced at the 4000 L scale.

### *How often will patients receive treatment and at what dose?*

Patients will receive the same dose and dose regimen used for routine treatment prior to the study.

### *How long will the study last?*

The primary study treatment period is for one year (52 weeks) followed by an extension period. The duration of time patients will be enrolled in the ADVANCE study is unknown at this time.

### *How much will the treatment cost while in the study?*

Infusion costs and treatment with the study drug, alglucosidase alfa (4000 L scale), will be provided at no cost for the duration of the patient's enrollment in the ADVANCE study.

### *Can patients be treated at home while in the study?*

The first four (4) infusions in the study will be conducted in the hospital or infusion center for observation of potential adverse events. Patients who meet specific criteria may have the option for home infusions thereafter and should speak with their physician to determine if this option is right for them.

### *Contact for more information:*

For additional questions about the ADVANCE study, please speak to your treating physician. You may also contact the patient organizations – the Acid Maltase Deficiency Association (AMDA) or United Pompe Foundation (UPF) - or your Genzyme case manager.