

Alglucosidase Alfa Temporary Access Program (ATAP)

Ages Eligible for Study: 18 Years and older

Genders Eligible for Study: Both

Criteria

Inclusion Criteria:

- The patient or patient's legal guardian must provide signed, informed consent prior to performing any study-related procedures.
- The patient must reside in the US.
- The patient must have a confirmed diagnosis of Pompe disease defined as documented acid alpha-glucosidase (GAA) enzyme deficiency from any tissue source and/or GAA gene mutations.
- The patient must have/had documented clinical signs and symptoms of Pompe disease.
- The patient must have/had prior treatment with alglucosidase alfa produced at commercial scale OR be naive to enzyme replacement therapy (ERT) for the treatment of Pompe disease and meet at least 1 of the following criteria: require a wheelchair OR require some respiratory assistance for any number of hours (including night time) through non-invasive ventilation.
- The patient must be capable of complying with the required program schedule of assessments.

Exclusion Criteria:

- Females who are pregnant or lactating
- The patient has a clinical condition unrelated to Pompe disease that would interfere with program assessments.
- The patient is currently enrolled in any clinical studies.

Locations

United States, Alabama

Sheffield, Alabama, United States

United States, Alaska

Anchorage, Alaska, United States

United States, Arizona

Phoenix, Arizona, United States

Prescott, Arizona, United States

Scottsdale, Arizona, United States

Tucson, Arizona, United States

United States, California

Bakersfield, California, United States

Beverly Hills, California, United States

Loma Linda, California, United States

Sacramento, California, United States

San Diego, California, United States

Stanford, California, United States

Los Angeles, California, United States

United States, Colorado

Aurora, Colorado, United States

United States, District of Columbia

Washington, District of Columbia, United States

United States, Florida

Bradenton, Florida, United States

Coral Springs, Florida, United States

Daytona Beach, Florida, United States

St. Petersburg, Florida, United States

Gainesville, Florida, United States

United States, Georgia

Decatur, Georgia, United States

United States, Idaho

Boise, Idaho, United States

United States, Illinois

Chicago, Illinois, United States

Hinsdale, Illinois, United States

United States, Indiana

Evansville, Indiana, United States

United States, Iowa

Iowa City, Iowa, United States

United States, Kansas

Kansas City, Kansas, United States

United States, Kentucky

Louisville, Kentucky, United States

United States, Louisiana

Shreveport, Louisiana, United States

New Orleans, Louisiana, United States

United States, Maine

Augusta, Maine, United States

United States, Maryland

Baltimore, Maryland, United States

United States, Massachusetts

Boston, Massachusetts, United States

Springfield, Massachusetts, United States

United States, Michigan

Lake Orion, Michigan, United States

Grand Rapids, Michigan, United States

St. Joseph, Michigan, United States

Detroit, Michigan, United States

Rochester Hills, Michigan, United States

United States, Minnesota

Minneapolis, Minnesota, United States

United States, Nebraska

Omaha, Nebraska, United States

United States, New Jersey

Midland Park, New Jersey, United States

United States, New Mexico

Albuquerque, New Mexico, United States

United States, New York

Albany, New York, United States

Corning, New York, United States

New York, New York, United States

Manhasset, New York, United States

United States, North Carolina

Durham, North Carolina, United States

Jacksonville, North Carolina, United States

Asheville, North Carolina, United States

United States, Ohio

Cincinnati, Ohio, United States

Cleveland, Ohio, United States

Toledo, Ohio, United States

United States, Oklahoma

Tulsa, Oklahoma, United States

Oklahoma City, Oklahoma, United States

United States, Oregon

Portland, Oregon, United States

United States, Pennsylvania

Philadelphia, Pennsylvania, United States

Pittsburgh, Pennsylvania, United States

United States, South Carolina

Greenville, South Carolina, United States

United States, Texas

Houston, Texas, United States

San Antonio, Texas, United States

Waco, Texas, United States

McKinney, Texas, United States

United States, Utah

Salt Lake City, Utah, United States

United States, Virginia

Charlottesville, Virginia, United States

Chesapeake, Virginia, United States

Newport News, Virginia, United States

Richmond, Virginia, United States

United States, Washington

Seattle, Washington, United States

Pullman, Washington, United States

United States, West Virginia

Morgantown, West Virginia, United States

United States, Wisconsin

Green Bay, Wisconsin, United States