Evaluating the safety of intravenous administration of enzyme replacement therapy in the home setting for infantile-onset Pompe disease

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BACKGROUND

Enzyme replacement therapy (ERT) is the only treatment for **Pompe disease**, a glycogen storage disease that causes debilitation in skeletal, cardiac, and smooth muscle. Without treatment, the disease progresses rapidly and most children die within the first year of life. ERT, specifically alglucosidase alfa, has demonstrated significant improvement in muscle involvement and improved long-term survival. Alglucosidase alfa is given intravenously over 6 to 8 hours every two weeks, and requires pre-medication. It carries a black-box warning due to potentially life-threatening cardio-respiratory complications during administration. For these reasons, it has typically been given in a hospital or clinic setting.

PURPOSE

The purpose of this evaluation was to determine if alglucosidase alfa could be safely administered in the home setting.

Recent studies have shown significant psychosocial benefits to families receiving ERT at home, including:

1. Increased flexibility
2. Fewer interruptions to patient routines
3. Decreased stress on the child, families, and workplace
4. Decreased expense of transporting patients (who are typically wheelchair and/or ventilator dependent) to the hospital/clinic

In addition, home infusion prevents exposure of medically fragile children to flu and other illnesses commonly transmitted in public settings like out-patient infusion centers.

METHODS

A multi-disciplinary approach was utilized in evaluating the safety of alglucosidase alfa infusions in the home. This home infusion organization's pharmacy worked with the referring clinic to develop criteria to screen patients for appropriateness for home care, including:

1. History of at least 12 months of hospital/clinic based infusions
2. No cardiac or respiratory compromise
3. Central vascular access
4. Compliance with treatment by patient/family

In addition, the clinic wrote a protocol for home administration of alglucosidase alfa, including:

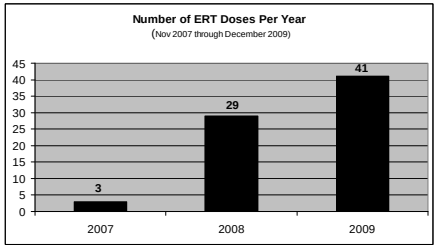
1. Dosing specifics
2. Monitoring parameters
3. Emergency protocols
4. Plan for coordination of care

There are only 17 Home Infusion Companies nation-wide that are administering alglucosidase alfa in the home setting.
(per pharmaceutical manufacturer)

The patients being considered for ERT had tracheostomies, ventilator support, and pulse oximetry which decreased concern for life-threatening reactions. This home infusion organization's nurses specialize in pediatric assessment, infusion therapy, and home care – all three skills are essential to safely manage these complex, medically fragile children during alglucosidase alfa infusions. Emergency kits containing epinephrine and other rescue medications are provided for all infusions. Additional training on Pompe disease and enzyme replacement therapy was provided to the infusion department by a representative of the drug manufacturer.

RESULTS

From November 2007 through December 2009, this home infusion organization administered a total of 73 doses of alglucosidase alfa to two patients in the home setting. These children are able to receive therapy with a minimal disruption to their day-to-day activities. No serious adverse events were seen during these infusions.



Total Doses Administered = 73

# of Severe Reactions = 0	**# of Other Reactions = 0**
Symptoms • Anaphylactic shock • Cardiac arrest • Cyanosis • Decreased oxygen saturation • Fever • Hypotension • Respiratory distress • Tachycardia	**Symptoms** • Agitation • Cough • Fever • Flushing • Headache • Hypertension • Hypotension • Irritability • Nausea • Rash • Tachypnea • Urticaria • Vomiting • Wheezing

In addition to the benefit for the patient, the decision to provide ERT in the home setting has also benefitted this organization. We have experienced an improved relationship with the referring physician/clinic, and have seen an increase in referrals for complex, multiple-therapy patients over the past two years. We now have three ERT patients on our service – two on alglucosidase alpha every other week, and one on laronidase every week.

CONCLUSION

Transition to the home setting for ERT infusions is possible with:

1. Careful patient selection
2. Reliable vascular access
3. Detailed management plan for adverse reactions
4. Comprehensive plan for coordination of care
5. Competent nursing staff with training in ERT and management of pediatric emergencies (if infusing ERT in children)

Ongoing management of these patients requires care coordination through close contact between providers, home infusion nurses and pharmacy, in-home extended hours nursing, and the family.

A success story: When we brought our first ERT patient home more than two years ago, his family had been encouraged to enroll him in a Hospice program. He was ventilated 24 hours/day, largely immobile, and had a poor prognosis. Today, because of his alglucosidase alfa infusions, he is a thriving three year old. He is currently ventilated only at night and lives an active lifestyle that includes going to school and a natural talent at playing guitar for his nurses!

DISCLOSURES

Authors of this presentation have the following to disclose concerning possible financial or personal relationships with commercial entities that may have a direct or indirect interest in the subject matter of this presentation:

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