Clinical Guideline

ENZYME REPLACEMENT THERAPY - MONITORING AND MANAGEMENT

SETTING
Bristol Royal Hospital for Children/Puzzlewood

FOR STAFF
All medical and nursing staff

PATIENTS
Patients < 18 years of age

Guidance

Enzyme replacement therapy (ERT) is currently available for an increasing number of lysosomal storage disorders. It is a lifelong therapy where patients receive weekly or fortnightly infusions of a foreign protein to which they may have hypersensitivity or immune mediated reactions. In most cases these can be easily managed.

ERT is initially administered in hospital but most patients will be transferred to home infusions in the absence of Infusion Related Reactions (IRRs) by three to six months.

Premedication; Most patients will initially be on premedication to reduce the risk of IRRs. This is usually oral paracetamol and an antihistamine (chlorphenamine, cetirizine, loratadine or hydroxyzine). Give 30 to 60 minutes before the ERT infusion.

For patients with mild to moderate IRRs, ranitidine may also be given and for patients with moderate to severe IRR low dose corticosteroids or disease modifying medications may be indicated.

Review and stop after ten weeks if there are no IRRs.

ERT infusions can also be unsettling. Patients may also benefit from

- Play therapy input
- Same nurse access with each infusion

Observations

Patients should have observations done – temperature (T), pulse (HR), oxygen saturations (SP O₂), respiratory rate (RR) and blood pressure (BP)

Before the infusion or premedication

- Perform observations and ask if the patient has been unwell or febrile. If there is a history of being unwell or observations are abnormal (e.g. Temp >37.3°C) please bleep the junior doctor (Bleep 6734) to do a clinical review before making up the ERT. ERT is very costly and every effort should be made to ensure that the infusion does not subsequently need to be discontinued. Consider postponing if acutely febrile or unwell. Junior doctors will discuss with the metabolic consultant on call so a decision can be made about continuing.

During and after the infusion

- Patients should initially have observations every 15 minutes for the first hour then every half hour. They should be monitored for any signs of IRRs (see table below).
- Observations should also be done before any increase in infusion rate (this would usually coincide with these frequencies).
- Post-infusion observe the patient for at least 30 minutes before allowing home. Patients who have had IRRs may be monitored for longer periods post infusion.
The frequency of monitoring can be reduced to half hourly observations after ten weeks if there are no IRRs.

## Management of IRRs

<table>
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<tr>
<th>Severity of symptoms</th>
<th>Management</th>
<th>ERT</th>
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| **Mild** | Check observations- HR, SP O₂, RR, T, BP  
  Clinical review by neurology juniors  
  Give po/iv antihistamine/paracetamol  
  Monitor patient to ensure symptoms settle. If not doctors to review for further management. | Slow infusion to half the rate  
  **Discuss all changes to management plan with metabolic consultant on call.** |
| **Moderate** | Check observations- HR, SP O₂, RR, T, BP  
  Clinical review by neurology juniors  
  Give po/iv antihistamine/paracetamol/ corticosteroid | Hold infusion  
  Clinician to consider restarting infusion at slower rate with slower increases in rate when reaction subsides.  
  **Discuss management plan with metabolic consultant on call.**  
  Observe in hospital for at least 1 hour post infusion.  
  **Subsequent infusions:**  
  Consider increasing premeds regime and giving infusion over a longer period |
| **Severe** | Urgent clinical review / 2222  
  Check observations- HR, SP O₂, RR, T, BP  
  Resuscitation  
  See guideline for [Anaphylaxis in Children](#)  
  Adrenaline/Antihistamines/paracetamol/corticosteroids | Stop infusion immediately  
  **Consult with metabolic consultant on call as soon as feasible**  
  **Subsequent infusions:**  
  Patient may need desensitisation in a controlled setting  
  Check drug antibodies. This will need to be coordinated with specialist biochemistry.(Ext 21299) |
### Table A

<table>
<thead>
<tr>
<th>REFERENCES</th>
<th>Related Documents and Pages</th>
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<tbody>
<tr>
<td></td>
<td>Anaphylaxis in Children</td>
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| AUTHORIZING BODY        | Metabolic Clinical Governance Group            |

| SAFETY                  | Naglazyme can cause congestive heart failure if patient susceptible to fluid overload |

| QUERIES AND CONTACT    | Contact department ext 21694 or metabolic consultant on call via switchboard. |