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### N&WICS - TA821 - Avalglucosidase alfa - Pompe disease

Before providing patient identifiable data on this form, please confirm that the patient (or in the case of a minor or vulnerable adult with the parent/legal guardian/carer) has given appropriate explicit consent for sensitive personal information on this form to be passed to the CCG and/or CSU for processing this funding request and validating subsequent invoices. Consent given: ☐

If there is more than one NICE-approved treatment available, a discussion between the responsible clinician and the patient has taken place about the advantages and disadvantages of the treatments available. This has taken into consideration therapeutic need and whether or not the patient is likely to adhere to treatment. The most appropriate, least expensive, will be chosen (taking into account administration costs, dosage and price per dose) unless an order of preference is stated in the TAs. ☐

<b>Patient NHS No:</b>	<b>Trust:</b>	<b>Practice Name:</b>
<b>Patient Hospital No:</b> <input type="text"/>	<b>Consultant Making Request:</b> <input type="text"/>	<b>Practice Postcode:</b>
<b>Patient's Initials and DoB:</b>		<b>Practice Code:</b>
<b>Notification Email Address:</b> <input type="text"/> (@NHS.net account ONLY)		<b>Contact name &amp; number:</b> <input type="text"/>
<b>Start date of requested treatment:</b> <input type="text"/>	<b>Provider:</b> <input type="checkbox"/> Private <input type="checkbox"/> NHS <b>Supplier:</b> <input type="checkbox"/> Homecare <input type="checkbox"/> Hospital	<b>Sub-Type:</b> <input type="text"/> N/A <input type="checkbox"/> (if applicable)

By completing this form, you confirm that you intend to use the requested medicinal product described below as agreed in the local commissioning statement. Any requests which fall outside of this use will require an individual funding request (IFR).

For support regarding IFRs, please contact: [norfolkicd@nhs.net](mailto:norfolkicd@nhs.net)

For support regarding the criteria listed below, please contact: [norfolknontariff@nhs.net](mailto:norfolknontariff@nhs.net)

Please indicate whether patient meets the following NICE criteria:	Please tick
<p>1. Avalglucosidase alfa (AVAL) is recommended, within its marketing authorisation, as an option for treating Pompe disease in babies, children, young people and adults, only if the company provides AVAL according to the commercial arrangement.</p> <ul style="list-style-type: none"> <li>Why the committee made this recommendation</li> <li>Pompe disease either occurs at birth (infantile onset; IOPD), or after 12 months (late onset; LOPD). The only treatment for Pompe disease is enzyme replacement therapy (ERT) with alglucosidase alfa (ALGLU). AVAL is an alternative ERT that works in the same way. Limited evidence shows AVAL can enter cells more easily, so reducing glycogen levels more efficiently than ALGLU. But the clinical benefit is uncertain.</li> <li>In LOPD, the cost-effectiveness estimates are uncertain because of uncertainties in the clinical evidence. But they are below what NICE normally considers an acceptable use of NHS resources, so AVAL is recommended for LOPD.</li> <li>Because IOPD is very rare, data is limited. So, assumptions about its efficacy were needed, which makes the cost-effectiveness estimates uncertain. When assuming that AVAL works as well as ALGLU, cost-effectiveness estimates are below what NICE normally considers an acceptable use of NHS resources. Given the high burden of Pompe disease on children and their carers, and the rarity of the condition, the committee accepted the uncertainties. So, AVAL is recommended for IOPD.</li> </ul>	<input type="checkbox"/> Yes <input type="checkbox"/> No
<p>2. As per NICE guidance, treatment should normally be started with the least expensive drug (considering the dose required, price per dose and any additional administration costs).</p> <p>Please confirm that the choice of drug is considered the most cost-effective treatment for this individual patient, noting that alternative treatment options recommended by NICE may be lower in cost, including the use of biosimilar medications if applicable.</p> <p>Where an alternative lower cost treatment is available and has not been tried by the patient, please confirm that the clinical rationale for prescribing has been included in the patients' medical record for the purpose of auditing.</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No

<b>I confirm that the patient meets the criteria for treatment</b>	
Name of person completing: <input type="text"/>	Contact Details: <input type="text"/>
Designation of person completing: <input type="text"/>	Date: <input type="text"/>
Trust Authorising Pharmacist	
Name: <input type="text"/>	
Date: <input type="text"/>	