

<b>A.33</b>	<b>Phosphorus – hypophosphatemic rickets – EMLc</b>
<b>Draft recommendation</b>	<input checked="" type="checkbox"/> Recommended <input type="checkbox"/> Not recommended Justification: Although the conditions that need the proposed medications are rare, yet it is currently still the recommended treatment for the condition. Therefore, the availability of these medications is essential for treatment of the conditions.
Does the proposed medicine address a relevant public health need?	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable Comments: The conditions that need the proposed medications are rare (X-linked hypophosphatemic rickets)
Does adequate evidence exist for the efficacy/effectiveness of the medicine for the proposed indication?  (this may be evidence included in the application, and/or additional evidence identified during the review process)	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable Comments: Number one on the list of treatment for the condition in the guidelines
Does adequate evidence exist for the safety/harms associated with the proposed medicine?  (this may be evidence included in the application, and/or additional evidence identified during the review process)	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable Comments:
Are there any adverse effects of concern, or that may require special monitoring?	<input checked="" type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not applicable Comments: Conventional treatment with phosphate supplementation and active vitamin D might increase calciuria and thereby promote nephrocalcinosis, which has been reported in 30–70% of patients with XLH. <sup>1</sup>

<sup>1</sup> Haffner D, Emma F, Eastwood DM, et al. Clinical practice recommendations for the diagnosis and management of X-linked hypophosphataemia. Nat Rev Nephrol. 2019;15(7):435-455. doi:10.1038/s41581-019-0152-5

24<sup>th</sup> WHO Expert Committee on Selection and Use of Essential Medicines  
Expert review

<p>Are there any special requirements for the safe, effective and appropriate use of the medicines?</p> <p>(e.g. laboratory diagnostic and/or monitoring tests, specialized training for health providers, etc)</p>	<p><input checked="" type="checkbox"/> Yes</p> <p><input type="checkbox"/> No</p> <p><input type="checkbox"/> Not applicable</p> <p>Comments: Oral phosphate supplements should always be provided together with active vitamin D (alfacalcidol or calcitriol, application A2), as phosphate alone promotes secondary hyperparathyroidism and thereby renal phosphate wasting. Patients with XLH (and other less common forms) should be followed by paediatricians and pediatric subspecialists. Pediatric endocrinologists are commonly those managing these patients.</p>
<p>Are there any issues regarding cost, cost-effectiveness, affordability and/or access for the medicine in different settings?</p>	<p><input type="checkbox"/> Yes</p> <p><input checked="" type="checkbox"/> No</p> <p><input type="checkbox"/> Not applicable</p> <p>Comments:</p>
<p>Are there any issues regarding the registration of the medicine by national regulatory authorities?</p> <p>(e.g. accelerated approval, lack of regulatory approval, off-label indication)</p>	<p><input type="checkbox"/> Yes</p> <p><input checked="" type="checkbox"/> No</p> <p><input type="checkbox"/> Not applicable</p> <p>Comments: Sodium phosphate is in the EML of 20+ countries (including some LMICs).</p>
<p>Is the proposed medicine recommended for use in a current WHO guideline?</p> <p>(refer to: <a href="https://www.who.int/publications/who-guidelines">https://www.who.int/publications/who-guidelines</a>)</p>	<p><input type="checkbox"/> Yes</p> <p><input type="checkbox"/> No</p> <p><input checked="" type="checkbox"/> Not applicable</p> <p>Comments: No WHO guidelines currently available for conditions which need the proposed medications.</p>