Product Type | Small molecule drug candidate / repurposed, reformulated
---|---
Indication/ROA | Harlequin ichthyosis (HI, orphan disease) and other ichthyoses, other lipid dysfunction disorders such as psoriasis; topical.
Target/MoA | Lead candidate modulates the ceramide-induced inflammatory responses in ichthyoses, improving keratinocyte differentiation and lipid barrier formation.
Development Stage | Lead candidate selected, efficacy demonstrated
Brief Description & Differentiation | Drug candidate improves the skin barrier dysfunction, inflammation and impaired keratinocyte differentiation that are a feature of lipid dysfunction disorders such as HI, a rare disease without effective treatment. HI is one member of a much larger disease family and a new treatment may have utility in other conditions such as psoriasis.  
- First-in-class topical anti-inflammatory drug - new ROA for a generic drug having long-term clinical use and a strong safety profile  
- Potential use of new formulation in other lipid dysfunction related skin indications  
- Potential for combination with retinoids  
- Other Anti-inflammatory drugs do not show the same effect
Research Team | Prof Ian Smyth and Dr Denny Cottle
Future | Develop commercial formulation, dosing and safety → 505(b)(2) regulatory pathway, orphan designation

**Key Data**
Efficacy in animal models – drug treatment reduced hydration, normalised differentiation of ichthyosis genes and induced skin thinning with no wounding or side effects.

### Key Data

<table>
<thead>
<tr>
<th>Drug</th>
<th>Aspirin</th>
<th>Actretin</th>
</tr>
</thead>
<tbody>
<tr>
<td>Reduced dehydration</td>
<td>✓</td>
<td>x</td>
</tr>
<tr>
<td>Improved differentiation</td>
<td>✓ ✓</td>
<td>x</td>
</tr>
<tr>
<td>Skin thinning</td>
<td>✓</td>
<td>x</td>
</tr>
<tr>
<td>No wounding/side effects</td>
<td>✓</td>
<td>x</td>
</tr>
</tbody>
</table>

- Improves keratinocyte differentiation
- Partially normalises Loricrin expression
- Partially restores Filaggrin expression