



Collaborative Working to Develop a Standard Worksheet for Preparation of a Gene Therapy Product

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Background

As part of the onboarding process for etranacogene dezaparvovec (Hemgenix[®]) a collaborative approach was agreed with NHSE, SPS Quality Assurance, Pan-UK ATMP Regulatory and Technical Subgroup and the 8 NHSE commissioned Gene Therapy haemophilia centres for a number of activities including the creation of a preparation worksheet.

- NHSE
- PAN-UK ATMP Regulatory and Technical subgroup
- SPS QA Lead
- Aseptic leads for the haemophilia centres involved
- Manufacturer via SPS QA lead

- Single pharmacist nominated led to draft the content
- Single pharmacist for each hub contributed /reviewed
- Teams meeting
- SPS approved

Working with and involving different stakeholders

Communication & Process

Pros

Collaborative Working

Cons

- Standardised approach to the dispensing of Hemgenix[®]
- Reduced workload for participating hospital sites
- Improved communication with the manufacturer by having a single point of contact asking the questions
- Improved communication with other stakeholders regarding the bigger picture timelines
- Input from centres who had experience with dispensing Hemgenix[®] as part of a clinical trial
- Input from centres who had experience with dispensing ATMPs
- Agree national deviation from SmPC – not to attach the giving set!
- Links with contract requirements
- Once agreed for local aseptic units to set-up on their worksheet / labelling system

- With standardisation loss of ability to fully customise or have bespoke worksheets
- Relying on a number of people to input into this piece of work, when it is outside the scope of their normal practice/job role leading to some slips in timelines
- Need a lead to start the process

First national collaborative worksheet - Hemgenix[®]

The worksheet is a detailed form for the preparation of Hemgenix. It includes sections for:

- Product Information:** Name, strength, and manufacturer details.
- Preparation Steps:** A series of numbered steps detailing aseptic techniques, such as 'Wash hands', 'Disinfect work surface', and 'Transfer product to the 500mL Sodium Chloride 0.9% w/v Intravenous Infusion (Patient weight - 220kg)'.
- Quality Assurance:** Checkboxes for 'Aseptic technique', 'Product inspection', and 'Final checks'.
- Signatures and Dates:** Fields for the preparer, checker, and approver, along with their respective dates.

The future

Pharmacy ATMP group have a process for developing other standardised worksheets for future treatments requiring onboarding at a national level. We can take this process and apply it at a more regional/local level for promoting standardisation or best practice for other ATMPs. This would reduce the set-up times for complex medicines, assist with capacity in S10 units and longer term reduce the training burden.

References:

¹Hemgenix SmPC CSL Behring UK Ltd ²Etranacogene dezaparvovec for treating moderately severe or severe Haemophilia B. NICE TA 989 24th July 2024
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