8.1: Aims and introduction

This chapter aims to describe how a proposed novel blood component, production process or blood pack is to be evaluated to:

- gain sufficient data to validate the component and production method
- gain sufficient data to support the clinical use of the component
- allow the Standing Advisory Committee on Blood Components (SACBC) to recommend to the Joint UKBTS/HPA Professional Advisory Committee (JPAC) that the component should be included in the Red Book
- provide sufficient information to prevent all Blood Transfusion Centres (other than those performing a full evaluation) from having to complete a full validation of the novel component before it enters routine production. They will only need to undertake installation and process qualification.

The chapter starts by identifying the steps that a group of investigators will need to undertake to submit a novel blood component for inclusion in the Red Book (see Table 8.1), thereby allowing it to be produced on a routine basis throughout the UK.

It is recognised that some novel components may be developed by a group of investigators in conjunction with a commercial company undertaking speculative research. As a result, the group of investigators may wish to enter the process at Step 9. In this case the SACBC will expect any requirements for data collection in the preceding steps to be complied with when the protocols and reports are submitted to the SACBC Chair for consideration. If sufficient data are not included then a request for extra data will be made (Step 11).

Guidance on how specific novel components should be tested is included in sections 8.2–8.4, and is followed by information on generic protocols for the evaluation of apheresis equipment (section 8.5) and blood packs (section 8.6).

Table 8.1 Steps for evaluation of novel components

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<th>Step</th>
<th>Details</th>
<th>Information</th>
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1. Investigators identify requirement for a novel blood component.

The requirement must be derived from R&D work or as the result of clinical discussions. The blood component needs:
- to fulfil an unmet clinical need
OR
- provide production benefit and have a Blood Service proposer.

Investigators will need preliminary data to support their application.

The new component may be derived from a commercially available product. In this case data to support the submission may be derived from the manufacturer.

Investigators must critically appraise data already available.

All data must be maintained on file. Data will be used to demonstrate validation has been completed in support of Blood Transfusion Centre licensing activities. Data required may include clinical outcome.

2. Investigators may obtain initial advice from the SACBC Chair as to whether the component should be treated as novel.

Yes: Go to Step 3.

No: Undertake local validation and produce the component locally under the general principles of good manufacturing practice and the Red Book.

The proposed new component may require evaluation even if it complies with existing Red Book guidelines if:
- a new production technique is involved (e.g. leucocyte-depleted red cells produced by apheresis)
- there are different steps in the production process (e.g. white-cell filtration immediately following collection).
- Definitive advice about the need for full-scale evaluation will be provided from the SACBC following a written submission.

Characterise the new blood component
3. Apply to the Standing Advisory Committee on Information Technology (SACIT) Chair for a development barcode (via the UKBTS Component Portfolio when available).

Allocated by the SACIT.

Allows component production, discard and use to be tracked using the Blood Transfusion Centre’s IT system. This will allow the evaluation to be integrated within the Centre’s quality system.

4. Investigators define the intended specification for the blood component.

Written specification to include:
- expected characteristics (e.g. leucocyte count)
- testing characteristics (blood grouping, microbiology etc.)
- sampling time, sampling method and sample handling conditions to confirm that the component meets specification.

Reference should also be made to the research papers from which the specification is derived.

Specify all key points which will allow subsequent production of the component to be well controlled.

5. Write the protocol for component evaluation.

Investigators’ group writes procedures for:
- component production
- monitoring of performance
- clinical use
- outcome measurement
- adverse incidents in production/use of the blood component

or uses manufacturer’s documentation to produce ‘in-house’ protocols.

Principles of good clinical and good manufacturing practice should apply. Comply with generic protocols (sections 8.2–8.5). Laboratory studies should comply with local standards.

Must include in the procedure the sampling regimes, data analysis and expected ranges, which will be used to confirm that production of the component is under control.

Must include detail of the data analysis methods.

6. Investigators should ensure their protocol complies with Chapter 8 and may seek advice from the SACBC.
7. Obtain ethics committee approval, if required.  
Must comply with local consenting and ethics policies for the use of donated material.

8. Investigators apply protocol.  
Document evidence of protocol being implemented.  
Investigation should be subject to independent quality audit.  
Audit may be carried out on behalf of collaborating manufacturers even though this may be confidential regarding the data collected. A summary outlining non-compliances against good clinical and manufacturing practice must be made available to the Blood Transfusion Service involved, for submission as part of the supporting documentation to the SACBC.

Obtain SACBC listing of the component

9. Investigators submit report and supporting data to the SACBC for consideration.  
Investigators review outcomes and produce a report, which summarises findings and supports the case for a new blood component to be listed.  
The SACBC decides if:  
- the blood component is novel  
- the data support the ability to produce the blood component on a regular basis  
- the blood component is efficacious and safe.  
Investigators who have been conducting speculative research with a manufacturer may enter the process at this point.  
This may also include data supplied by manufacturers, other Blood Services and published studies.  
Investigators should submit a draft specification for the component.

10. The SACBC decides whether the component may be recommended for inclusion in the Red Book guidelines.  
If the SACBC decides that the blood component will be listed, it submits this recommendation to the Red Book JPAC.  
If the SACBC decides that the blood component will not be listed, it informs the submitting group and provides an explanation.  
The SACBC may request further data in support of the submission prior to listing the blood component.

Joint Professional Advisory Committee
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<thead>
<tr>
<th>Number</th>
<th>Task Description</th>
<th>Details</th>
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<tbody>
<tr>
<td>11.</td>
<td>Consider the recommendation that a new component should be listed.</td>
<td>Write to the SACBC notifying it of the decision. If not accepted, provide the SACBC with detailed reasons for the decision.</td>
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<td><strong>SACBC</strong></td>
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<td>12.</td>
<td>Communicates the JPAC decision to appropriate parties.</td>
<td>If accepted inform investigators and request the SACIT to proceed with the provision of appropriate labels. Write to Medical Directors of the four UK Blood Transfusion Services. Provide copies of the data and report used to accept the new blood component. If not accepted inform investigators, with supporting reasons.</td>
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<td><strong>SACIT</strong></td>
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<td>13.</td>
<td>Provides codes for the new blood component.</td>
<td>Code will be unique. ISBT 128/ABC Codabar will be supported.</td>
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<td>14.</td>
<td>Provides a component label and updates the UKBTS Component Portfolio.</td>
<td>Label will be unique. Label text will describe the key attributes of the component.</td>
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<td><strong>Blood Establishment</strong></td>
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<td>15.</td>
<td>Begin production of the new blood component.</td>
<td>Base procedures on those used during validation studies. Complete installation and process qualification. Demonstrates without redoing the above validation that the blood component produced is equivalent to that defined in the UK guidelines.</td>
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