Case Study: developing the resource *ATMPs: A guide to preparing for Health Technology Assessment in the United Kingdom*

The Challenge

As the market for Advanced Therapy Medicinal Products (ATMPs) is still relatively immature, best practice around preparing for health technology appraisal (HTA) and the unique challenges that these medicines may bring within the context of the national assessment framework are not yet widely understood. However, if submissions to HTA bodies are to be robust and meet adoption requirements, it is critical that ATMP manufacturers understand and anticipate the challenges relating to generation of an optimal evidence package for their products. A lack of strategic planning, which will proactively overcome such challenges, may ultimately result in failure to demonstrate the value of their products to HTA agencies, even if they are both clinically and cost effective. This outcome could have a significant impact on the commercial viability of the ATMP developer and deprive NHS patients of the benefit these treatments could represent.

The Solution

A key objective of the Northern Alliance Advanced Therapies Treatment Centre's (NA-ATTC) programme is to support ATMP developers and NHS Trusts and Boards accelerate the adoption of these innovative medicines. In part, this can be achieved by providing guidance on regulatory, health economy and commissioning processes and pathways for ATMPs. It was envisaged that best practice information be developed in the form of an online document, aimed at small and medium biotechnology companies working in the field of ATMPs, relevant University Departments and NHS Blood and Transplant services. This guidance is designed to ensure submissions to HTA bodies by ATMP developers are as robust as possible and meet medicine adoption requirements. It will also add to the growing repository of information on ATMPs, assisting with easing the burden of their adoption into clinical practice.

The Results

Experts from NA-ATTC, including Chiesi Limited, Bresmed Health Solutions, University of Leeds and the Cell and Gene Therapy Catapult produced *ATMPs: A guide to preparing for Health Technology Assessment in the United Kingdom*. This comprehensive resource provides guidance on best practice for developers regarding quality of life approaches for ATMPs, a health economic framework for ATMPs and guidance for how developers ensure their products are identified in horizon scanning processes by HTA bodies.

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The guide includes:

- An overview of the Regenerative Medicine Expert Group subgroup for evaluation and commissioning
- Considerations for clinical study design of ATMPs
- Regulatory approval and beyond
- Horizon scanning
- HTA considerations for assessing ATMPs
- Principles of health economic review
- HTA submission considerations

This publication is available on the ATTC website via: NA-ATTC-HTA-Guide-fv.pdf

Making further impact

The guide has been extensively publicised; networks such as The Association of the British Pharmaceutical Industry (ABPI), BioIndustry Association (BIA), Advanced Therapy Medicinal Products Manufacturing Community (AMC) and the ATTC Industry Advisory Group (IAG) have been part of the dissemination drive to make companies aware this resource can support their HTA journeys. In parallel, NA-ATTC IAG colleagues have contributed to the recent consultation reviewing NICE's process for health technology evaluation.

HTA agencies across the UK

Key: AWMSG, All Wales Medicine Strategy Group; HSC, The Health and Social Care Board; NICE, National Institute for Health and Care Excellence; SMC, Scottish Medicines Consortium; arrows indicate explicit influence of recommendations under certain circumstances

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