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23 September 2021

Dr. June Raine
MHRA
10 South Colonnade
London
E14 4PU
United Kingdom

Re: Consultation on Point of Care Manufacturing

Dear Dr. Raine,

On behalf of the International Society for Stem Cell Research (ISSCR), I write to share our comments on the proposed regulatory framework for point of care manufacturing. The ISSCR is the leading professional organization of stem cell researchers and represents more than 4,000 members in the United Kingdom and around the world. Our members are scientists, clinicians, ethicists, and educators dedicated to the responsible advancement of stem cell research and its translation to the clinic. While we support advances in the manufacturing of cell therapies that can safely increase access to advanced cellular therapies, any new regulatory framework for medicines manufactured and supplied at the point of care must reinforce current safety, quality, and efficacy standards and safeguard consumers from unproven products.

Relative to allogeneic therapies, autologous cellular therapies reduce the likelihood of immune rejection and eliminate the need for lifelong immunosuppressants. The localized manufacturing and distribution of autologous cellular therapies have the potential to simplify product development compared with similar products that require transporting a patient's cells to and from a centralized manufacturing facility. We appreciate that the proposed regulatory framework for point of care manufacturing has the potential to facilitate the development and delivery of safe and effective autologous cell therapies by streamlining the oversight of this process. However, the structure of a new regulatory framework for point of care manufacturing must ensure that products, like Advanced Therapy Medicinal Products (ATMPs), are held to the current robust standards for safety, quality, and efficacy. We are concerned that the consultation document groups broad categories of products together, including ATMPs, blood products, and medicinal gases. Due to the complexity of ATMPs, which often entail the manipulation and non-

Promoting excellence in stem cell science and applications to human health.



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homologous use of cells, including gene editing and the creation of derivatives, ATMPs may warrant special consideration and separate regulations for point of care manufacturing to ensure their quality, safety, and efficacy.

We are also concerned that unscrupulous businesses and clinics seeking to capitalize on consumer excitement for regenerative therapies may seek to exploit unintended loopholes in the new regulatory framework for point of care manufacturing to sell unproven cellular therapies. As we noted in [a letter last year](#), the surge of these businesses puts patients at physical and financial risk and undermines the development of legitimate new therapies. Unproven treatments marketed by these businesses have resulted in patients being blinded in the [UK](#) and [US](#), [paralyzed](#), and infected with [dangerous pathogens](#). Therefore, any regulatory framework designed to encourage innovation and facilitate the local manufacture of ATMPs and other medicinal products must be scrutinized to ensure it does not have unintended consequences or create loopholes that enable unscrupulous businesses to evade regulatory oversight. We are particularly concerned that purveyors of autologous adipose-derived cell therapies might seek to exploit the regulations to sell adipose-derived cells for indications that they have not been shown to treat effectively. These therapies and all ATMPs should be preceded by well-regulated trials and testing that demonstrate the safety, efficacy, and quality of such products.

Thank you for considering our views on point of care manufacturing for cellular therapies and other ATMPs. If the ISSCR can clarify any of these views or be of assistance, please contact Eric Anthony, ISSCR's Director of Policy at eanthony@isscr.org.

Sincerely,

Melissa H. Little
President, ISSCR
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