Annex A – Legal basis and the assessment of the matters set out in section 2 of the Medicines and Medical Devices Act 2021

The Medicines and Medical Devices Act 2021 ('the Act') received Royal Assent on 11 February 2021. We propose to make the legislative changes under consultation in this document, under Part 2 of the Act, which provides powers to make regulations about human medicines.

This consultation is conducted in line with the consultation requirement in section 45(1) of the Act.

Section 2 of the Act states that safeguarding public health must be the overarching objective of the appropriate authority when making regulations. Section 2 requires that when assessing whether regulations would contribute to the objective of safeguarding public health, the appropriate authority must have regard to three factors:

- The **safety** of human medicines
- The **availability** of human medicines
- The likelihood of the relevant part of the United Kingdom being seen as a **favourable place** in which to carry out research relating to human medicines, conduct clinical trials, or manufacture or supply human medicines

We have assessed the proposals against each of these factors, outlined below.

Updates to clinical trial application processes and approvals: We are proposing a range of process changes, which will simplify and streamline application processes, update requirements for Research Ethics Committees and update UK definitions. This will ensure regulations reflect current practice, reduce burden on those conducting trials and remove legislative blockers to innovation.

- **Safety:** It is critical that the safety of those participating in clinical trials is at the centre of clinical trials. Whilst some proposals such as enabling combined ethics and regulatory approvals will simplify and speed up overall timelines for approval, trial applications will undergo the same stringent assessments, we do not consider the changes proposed to streamline trial processes will have a negative impact on patient safety. Trials will still be held to the highest relevant requirements and safety standards, so trial participants can be assured of their safety while taking part in UK trials.
- **Availability:** Proposed process updates will also include clarifying requirements for collection of real-world data. This will open up the great

potential for the use of real-world data to increase the speed and reduce the cost of development programmes. This could see effective medications being approved more quickly, or even enable research programmes which were previously thought to be unfeasible.

- **Favourability:** Updated, streamlined processes will support more rapid timelines for overall trial approval than via separate application, submission and review by MHRA and ethics, increasing the attractiveness of the UK for researchers. We will be able to process and approve clinical trial submissions in a much faster and more proportional and pragmatic way. We are also proposing to modernise UK terminology and promote international harmonisation of definitions. This will support international trials continuing in the UK, which will be of particular importance for trials where international collaboration is critical, for example trials for rare diseases.

Public involvement and transparency: we are proposing to introduce requirements into legislation to ensure information about clinical trials is publicly available from the beginning to end of a trial, and for patient involvement in trial set up from the early stages.

- **Safety:** These proposals align with the Health Research Authority "Make it Public: transparency and openness in health and social care research" strategy and recommendations in the Independent Medicines and Medical Devices Safety Review for transparency and for patients to be at the centre of regulations. For instance, we propose legislating to require public involvement in trial design and implementation will provide a valuable mechanism through which patients are able to raise any safety concerns. We consider these changes will help provide for a clinical trials environment that is more patient centred, ensuring trials are in the best interests of patients and the public.

- **Availability:** We do not anticipate the proposals for transparency and patient involvement would directly affect availability of medicines.

- **Favourability:** We recognise that new requirements could place a burden on those undertaking clinical trials, however transparency about what research is going on and its findings benefits the research community as a whole. Volunteers participating in clinical trials generously give their experience and time to help advance treatments; ensuring patients are the centre of trial design could encourage more volunteers in future trials. Ensuring greater transparency about clinical research and medicines development allows researchers to learn from each other, promoting an open and collaborative UK clinical research environment which will encourage further research and trials to be conducted in the UK.

Safety reporting: We are proposing to remove reporting requirements that add burden to investigators but do not contribute to participant safety.

- **Safety:** Safety reporting requirements provide that the MHRA has the necessary regulatory oversight to ensure the upmost protection of safety of trial participants. In some instances, reporting requirements add a burden to trial sponsors, without giving added assurance of patient safety, for example where there are duplicative reporting requirements. We consider that removing these will not impact patient safety because the information is already received through another route. Critical reporting requirements will not change, ensuring participant safety is not compromised. The changes proposed will make sure that meaningful information is available to investigators and regulators in order to ensure appropriate actions are taken to protect the safety of trial participants.

- **Availability:** We do not anticipate the proposals for safety reporting would directly affect availability of medicines.

- **Favourability:** Removing unnecessary requirements on sponsors to report, where possible without impacting on safety, will reduce burden on trial sponsors. This will make running trials in the UK easier, promoting the UK as an attractive place to run trials.

Good Clinical Practice and corrective measures: We are proposing to provide flexibilities to enable more proportionate regulation of lower-risk trials, whilst ensuring proportionate sanctions and corrective measures.

- **Safety:** Different trials have different levels of risk, and regulatory requirements need to be proportionate, whilst still ensuring patient safety. For example, a trial for a completely new medicine that has not been used in people before would be high risk, whilst a medicine that is in wide use and already demonstrated as safe in the trial population would be lower risk. Embedding a proportionate approach will reduce unnecessary burdens to trial sponsors whilst ensuring compliance with requirements that are necessary to ensure safety of the trial product and protect trial participants. We are also proposing additional corrective measures, enabling the MHRA to make a decision on applications for a new trial taking into account any on-going evidence of serious GCP non-compliance. This will increase our regulatory oversight, meaning we can better ensure safety of participants in new trials.

- **Availability:** We do not anticipate the proposals for safety reporting would directly affect availability of medicines.

- **Favourability:** Embedding principles of risk-proportionality in legislation offers much greater clarity to those conducting clinical trials and give sponsors the confidence to employ these regulatory flexibilities. This has the potential to reduce burden and complexities for sponsors conducting lower risk trials. We recognise that taking products through clinical trials requires regulatory support and guidance, the MHRA offers informal and formal regulatory and

scientific advice and will continue to encourage and support novel methodologies and new ways of working. This will help support our proportionate and supportive regulatory environment, to further encourage sponsors to conduct trials in the UK.

Manufacturing and assembly: We are proposing some changes to introduce proportionate requirements for the labelling of products manufactured or imported for clinical trials, and specifically for diagnostic radiopharmaceuticals.

- **Safety:** Proposals are intended to reduce unnecessary burden, however the safety of products being imported or manufactured for use in clinical trials will not be compromised. The high standards required to demonstrate safety of a product to be used in a trial will not change.

- **Availability:** We are proposing to provide an exemption for holding a Manufacturers Authorisation for IMPs (MIA(IMP)) for preparing radiopharmaceuticals used as diagnostic IMPs. This will reduce the administration needed to use these products in trials, which will therefore support availability in hospitals, health centres and clinics for use in clinical trials. Whilst exempt from requiring an MIA(IMP), radiopharmaceuticals used in a clinical trial would still be subject to the requirement to be manufactured to an appropriate level of Good Manufacturing Practice, e.g. at a site holding a Manufacturers Specials licence, which will ensure they still meet necessary quality, safety and efficacy requirements.

- **Favourability:** Proposals will reduce burden and make it easier when importing products into the UK for trials e.g. in the labelling of products, this will further promote the UK's attractiveness to trial sponsors.