Annex A: Engagement Questions

Section 1: Background and Purpose and **Section 2:** Introduction	Strongly agree	Agree	Neither agree nor disagree	Disagree	Strongly disagree	Don't know / NA
a. Do you agree with purpose of the Innovative Medicines Fund?	❑	❑	❑	❑	❑	❑
b. Do you agree that the Innovative Medicines Fund should operate alongside, and on similar terms to the Cancer Drugs Fund?	❑	❑	❑	❑	❑	❑
Comments:	In general terms, the Blood Cancer Alliance welcomes the introduction of the Innovative Medicines Fund. The Cancer Drugs Fund has served to improve patient access to innovative cancer treatments, and it is only fair that patients with non-cancer diseases have access to innovative treatments on equitable terms. Managed access schemes are a vital method of speeding up access to effective new treatments for UK patients, and for encouraging innovation and inward investment in the UK life sciences sector. We note that, while similar, the IMF is not identical to the CDF in terms of its framework and principles. We ask for clarity as to whether there will a future exercise in standardising the CDF framework and principles with the new IMF framework and principles. The funding that will be made available to the IMF is exactly equal to the funding available to the CDF. While in general we support the level of funding available under IMF, we would also ask for clarity of evidence underpinning the need for the amount to be exactly equal, in terms of the analysis of treatments in the pipeline, or analysis of the number of NICE appraisals that have seen significant uncertainty that could adequately be addressed via a managed access scheme in both cancer and non-cancer. In this context, we would also ask whether the allocation of resource of the IMF means that there will be no scope for increase in available resource for the CDF in the short to medium term? The Blood Cancer Alliance strongly recommends that decisions on necessary resource for each of					

	these two schemes are taken wholly independently from one another, and based on clear evidence of a robust estimate of the number of innovations that will benefit from managed access schemes for both. This will ensure that, the CDF has adequate resource to make provision for new cancer innovations for the future. In order to achieve transparency in this area, we would ask that NICE and NHS England publish figures for the annual rebate or underspend for the IMF, in line with current practice for the CDF. In paragraph 13 of the IMF consultation document, the term 'reasonable price' is included. We would welcome a definition of how NICE and NHS England will assess what is a 'reasonable' price. In the absence of a clear and consistent definition, we urge this language to be changed to reference to 'cost-effective new medicines and treatments', given the clear precedent for cost effectiveness being an existing and accepted evidence-based assessment within the current NICE appraisal processes We welcome the strong focus on data collection within the IMF outlined in paragraph 15. However, the Blood Cancer Alliance's 2020 Report, Access to Medicines, found evidence that within the CDF that data to address uncertainties identified in the NICE appraisal process is not always being collected. We urge that Data Collection Agreements (DCAs) pursued under the IMF are clear not only on what data needs to be collected to address uncertainties, but who is responsible for data collection and how it will be facilitated. Should evidence emerge that issues of data collection prevalent under the CDF are not being experienced under IMF, we ask that an effort is made to standardise DCAs across the two schemes. On a final note, we ask that DCAs are shared with patient organisations in order to facilitate transparency in the data collection process.

Section 3: Guiding principles for the Innovative Medicines Fund	Strongly agree	Agree	Neither agree nor disagree	Disagree	Strongly disagree	Don't know / NA
a. Do you agree with the objectives and guiding principles underpinning the Innovative Medicines Fund?	❑	❑	❑	❑	❑	❑

Comments:	Principle One: We do not support the inclusion of the terminology ‘uncertain medicines’. It is often clear that medicines that require managed access schemes to address uncertainties are better than the current standard of care, in relation to patient outcomes, but that there is simply not enough data for these medicines to complete the standard NICE appraisal process. Principle Two: The explanatory note for this principle highlights that medicines must demonstrate significant clinical benefit for inclusion in the IMF. We ask for clarification on the thresholds for determining this, but also highlight that it is at odds with the point we raise in terms of the terminology used in Principle One. A medicine cannot be both uncertain in it’s benefit and be of significant clinical benefit. Principle Three: We are concerned by the inclusion of a requirement of consideration of NHS administrative issues such as data collection resource as a component of discussions on price, and therefore availability of treatments via the IMF. There is a potential that bringing additional factors over and above cost-effectiveness into pricing considerations will deter industry from utilising the IMF route, and therefore stifling innovation and denying patients cutting edge new treatments. Principle Four: Given that some medicines that will be made available via the IMF will be for patients with rare diseases, we do not support a fixed time limit on IMF availability, as it may take longer than five years to collect the volume of data necessary to address uncertainties, due to small patient numbers. Instead, we would support a principle of treatments being available for a period to allow adequate data collection to address uncertainties and facilitate a successful future full NICE appraisal. This is particularly important given that the NICE Methods Review process has not led to a rarity modifier being introduced to the full appraisal process. Principle Five: We support the principle of IMF treatments being made available to the whole eligible patient cohort, as is the case with the CDF.

Section 4: Key Features of the Innovative Medicines Fund	Strongly agree	Agree	Neither agree nor disagree	Disagree	Strongly disagree	Don’t know / NA

To what extent to you agree with the following key features of the Innovative Medicines Fund?						
a. NICE recommending a medicine in the Innovative Medicines Fund?	❑	❑	❑	❑	❑	❑
Comments:	Para 24: We would like to understand what criteria, if any, outside cost-effectiveness will be considered in the case for adoption? It is not clear within the key features that cost-effectiveness will be the only criteria applicable. Para 25: We would welcome clarification on whether the DCA needs to be in place before treatments are made available to patients under the IMF. DCAs can often take a significant time to agree, and waiting may cause a delay to patients being able to benefit from innovative new treatments.					
b. Criteria for entry into the Innovative Medicines Fund?	❑	❑	❑	❑	❑	❑
Comments:	n/a					
c. Resolving uncertainty through the Innovative Medicines Fund?	❑	❑	❑	❑	❑	❑
Comments:	Para 28: We would welcome clarity as to how NICE intends to share information on identified uncertainties with stakeholder such as patient organisations. This is critical to transparency and accountability within the process. Para 29: We welcome the commitment to involve stakeholders in the process of developing the DCA. We urge that there is clear guidance to stakeholders as to what the opportunity is to contribute, and how the process will work. This is not a feature of the CDF and we also urge consideration of how these practices can be applied to that managed access scheme. Para 30: We welcome that the DCA will be subject to regular review, and urge transparency of this process with patient representative organisations. Again, we strong suggest this process is also applied to the CDF in future to address challenges of transparency and data collection in that scheme that have been identified by the Blood Cancer Alliance. Para 32: We note the expectation that companies will pay a proportion of the costs associated with data collection. In the principles section of the document, it is also suggested that the price of the treatment reflect the ‘burden’ of data collection,					

	which we can only assume is a resourcing issue. The Blood Cancer Alliance is concerned that the perception that companies are being asked to effectively pay twice for data collection may disincentivise them from bringing innovative new treatments to the UK for appraisal that may facilitate progress via the IMF. Instead, we urge NICE and NHS England to be clear on how companies will be expected to contribute to data collection resource required in a transparent way, in order to ensure there is no deterrent to companies making innovations available to UK patients via this route.					
d. Commercial Access Agreements (CAA)	❑	❑	❑	❑	❑	❑
Comments:	Para 40. We would like to see further clarity on this statement regarding the range of ICERs in the cost-effectiveness range at entry to the IMF. It is not clear how many of the ICERS would be considered plausibly cost effective – this should be defined. By adding “as a minimum” compared to CDF guidance, this suggests the NHSE routinely expects IMF treatments to be offered at a price over and above the normal range of cost-effectiveness. We are concerned that the level of discount that would be required will mean pharmaceutical companies are disincentivised to access the fund. Para 44. We would welcome clarification as to the process if a company decides to propose a new reimbursement mode. In particular, we would like to understand whether the full appraisal process will be initiated from the very beginning, or whether such treatments can go to the latter stages of price appraisal, thus limiting any delay to them being made available to patients. We do not challenge the charging of the full appraisal fee to companies, but would support an expedited process in order to benefit patients from new innovations as soon as possible.					
e. Updating NICE guidance following a period of managed access and exiting the Innovative Medicines Fund?	❑	❑	❑	❑	❑	❑
Comments:	Para 57: We do not support that treatments can not enter the IMF again at a later date, particularly given the time limit of five years and the challenges involved in data collection for treatments for rare diseases.					
f. Interim Funding for NICE recommended medicines?	❑	❑	❑	❑	❑	❑
Comments:	Para 60. We would welcome more information as the influencing factors and the kinds of timeframes that would be applicable in this situation, so that we can understand the implications for patients.					
g. Financial control?	❑	❑	❑	❑	❑	❑
Comments:	*Please provide any further comments you have here.*					

Section 5: Conflict of interest disclosures: have you or the organisation you represent received any payments, grants or other funding from the pharmaceutical and life science industry in the last three years?

☐ Yes ☐ No

If yes, please specify the source of funding and sums involved in each of the last three years:

2018/19 funds raised (£)

Janssen Donation	30,000
Total	**£30,000**

2019/20 funds raised (£)

Janssen donation	15,000
Gilead donation	25,000
Pfizer donation	7,000
Novartis donation	20,000
Amgen donation	5,000
Kyowa Kirin donation	7,500
Sanofi donation	15,000
Takeda donation	12,500
Celgene donation	25,000
Incyte donation	15,000
Total	**£147,000**

2020/21 (£)

Janssen donation	15,000
Gilead donation	20,000
Novartis donation	12,500
Amgen donation	10,000
Kyowa Kirin donation	15,000
Takeda donation	15,000
BMS donation	12,500
Incyte donation	15,000
Abbvie donation	15,000
Roche donation	15,000
Total	**£145,000**

.

Section 6: Please tell us which organisation you work for/are responding on behalf of:

Blood Cancer Alliance