SANOFI RESPONSE

Proposed Changes to TGA's Orphan Drug Program

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INTRODUCTION

Sanofi welcomes the opportunity to provide further input into the proposed changes to the existing Orphan Drugs Program, having provided feedback into the initial 2015 consultation as part of the Medicines Australia response. The following Sponsor commentary is provided to assist with discussion as part of the next round of this consultation:

QUESTION 1: RARE DISEASES THRESHOLD vs. LACK OF FINANCIAL VIABILITY AND SERIOUSNESS OF THE CONDTION (CRITERION 1)

Sanofi wholeheartedly agrees there is need to raise the current threshold to make it less restrictive to bring it in line with the approach taken by other tier 1 regulators.

We consider that a model that allows for future increases in population better serves Australian patients and that that the proposed 5/10,000 threshold is appropriate.

We are also in agreement with the proposal to apply Orphan Drug Designations to medicines used to treat life threatening or chronically debilitating conditions that exceed the proposed thresholds but are nonetheless small enough in number to be not financially viable.

QUESTION 2: ALTERNATIVE METHODS OF DIAGNOSIS, PREVENTION AND TREATMENT (CRITERION 2)

Sanofi considers that the proposed criteria are appropriate and would anticipate that TGA designation would be aligned with the determinations of comparative regulators.

QUESTION 3: MEDICAL PLAUSIBILITY (CRITERIA 3 AND 4)

In line with the spirit of the current orphan Drug Program, Sanofi agrees that proposed patient populations for orphan conditions need to be well characterized. Where use in a particular sub-group of patients is proposed, it would be appropriate to request justification as to why the proposed therapy could not be used in a broader population, prior to designation.

QUESTION 4: PAEDIATRIC INDICATIONS

Sanofi is pleased to note the TGA comment that proposed changes are expected to increase the number of paediatric conditions that could receive Orphan designation. We are also in agreement with the current and proposed requirement that prevalence criterion are applied to the whole of the disease, or to specific paediatric sub-groups where applicable. As part of the designation approval for paediatric indications, sponsors should continue to provide a specific rationale as to why use

of the therapy is applicable to that specific age group, and importantly, why use of that therapy is inappropriate in other age groups.

QUESTION 5: DESIGNATION PROCESS AND AUTOMATIC LAPSING

We are in general agreement with the proposed designation process as described and consider that the proposed 3-6 month period in which to submit for registration following Orphan designation is appropriate in the majority of cases, with the option for extension with justification in exceptional circumstances.

In cases where designation has been granted and subsequently revoked following to registration of another therapy for that same condition, our expectation is Sponsors that have already submitted for registration under designation would not be charged retrospectively for evaluation fees.

QUESTION 6: OTHER KEY ISSUES

Where the Sponsor is proposing alternatives to the arrangements proposed in the consultation document, these have been provided with the corresponding responses.