الهيئة العامة للغذاء والدواء
Saudi Food & Drug Authority

BREAKTHROUGH MEDICINE PROGRAM

Version 1.0

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Saudi Food & Drug Authority

Drug Sector

For Inquiries *SDR.Drug@sfda.gov.sa*

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Please visit SFDA's website at
https://www.sfda.gov.sa/en/regulations?tags=2

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Saudi Food and Drug Authority

Vision and Mission

Vision

To be a leading international science-based regulator to protect and promote public health

Mission

Protecting the community through regulations and effective controls to ensure the safety of food, drugs, medical devices, cosmetics, pesticides and feed

Document Control

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1. Introduction

Breakthrough medicines program aims to facilitate and accelerate development and review of new drugs that address unmet medical need in the treatment of serious or life-threatening conditions. The program is voluntary and based on enhanced interaction and early dialogue with drug developers, to optimise development plans and speed up evaluation and ensure that promising medicines are available as soon as it can be concluded that the medicines' benefits justify their risks.

Under the breakthrough medicine program, the designation request may submitted by the end of phase 2 or at any time after. The SFDA will assess the application and provide a guidance based on the stage of drug development and provided data at submission time.

2. Related documents

This document should be read in conjunction with the following drug sector documents:

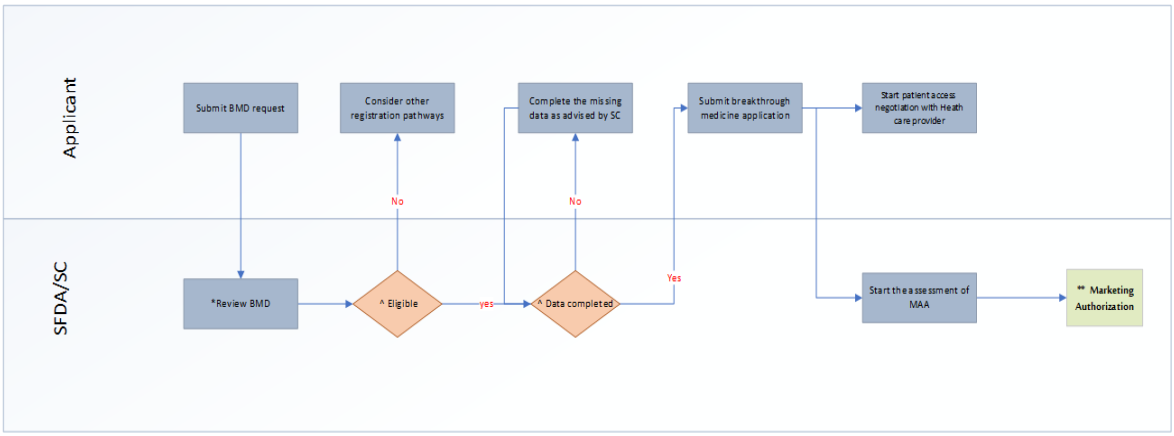
- The Data Requirements for Human Drugs Submission.
- GCC Module 1 Specifications.
- Guidance for submission
- Regulation and Requirements for Conducting Clinical Trials on Drug
- Any document issued by the SFDA in this regard.

3. Eligibility Criteria

All four criteria must be fulfilled in order to gain a breakthrough medicine designation are:

1. Target serious debilitating or life-threatening conditions with unmet medical need.
2. The medicinal product is likely to offer major advantage over methods currently used.
3. The potential adverse effects of the medicinal product are considered to be outweighed by the benefits, allowing for the reasonable expectation of a positive benefit/risk balance.
4. The product is not registered at any regulatory authority at the time of submission of the designation request.

4. Procedure for breakthrough medicine designation and patient access



*Pre-designation Meeting might be requested
^ this step is done jointly with scientific committee
** official medicine access after granting marketing authorization
BMD = breakthrough medicine designation ; SC= scientific committee ; MAA=marketing authorization application

1. Submission of breakthrough medicine designation request:

- Applicant should submit an eligibility request for breakthrough medicine designation via SDR.drug@sfda.gov.sa, using the breakthrough medicine designation form (See appendix 1).
- Applicants are encouraged to inform the SFDA whether they have submitted a request for designation or eligibility to the other agency and the outcome of this request.
- SFDA might request a pre-designation meeting to discuss matters related to the quality, safety and efficacy of the medicine.
- The scientific committee will review the application (the review normally takes 30 working days of the receipt of your request). The focus of the review is restricted to (1) the potential of the medicinal product to fulfil the eligibility criteria, and (2) the format of the data to be submitted, data availability, and readiness of the marketing authorisation application.
- Following the review, the scientific committee will decide on whether to grant a designation or not and the applicant will receive an email on the committee's decision.

Note:

– In case of rejected designation request, the applicant can utilise the SFDA's scientific advice service or may consider other registration pathway.

- Breakthrough medicine designation may be withdrawn if emerging data were to show that the criteria are no longer met.

2. Based on the completeness of the data, the applicant will be directed to one of the following:

Case 1: Completed data (no need for further data collection):

- Applicant must submit the medicine application file within 30 working days after granting the designation.
- Application shall be submitted through Saudi Drug Registration (SDR) System and according to the "Guidance for Submission".
- Applicant may request an exemption for one or more of the registration requirements, if unavailable (e.g. CPP, leaflet and artwork)
- Assessment of breakthrough medicine application:

1. The scientific assessment takes 60 working days; SFDA may send a set of inquiries to the applicant.
2. In case of inquiries, the applicant shall respond to SFDA's inquires within 60 working days (this may be extended in exceptional circumstances).
3. When the SFDA has sufficient information about the product, it will make a decision on whether or not a medicine should be granted a marketing authorisation.

Note: the company can start the medication access negotiation process with all involved healthcare entities in parallel with application review.

Case 2: Incomplete data (required to provide more data)

- Applicant that grant breakthrough medicine designation will be required to provide more data when the currently available data related to drug development is insufficient to make a decision and may require further clinical trials and collecting more data.
- Scientific committee will conduct an assessment on the submitted data and provide scientific advice to help the applicant design and conduct a drug development program as efficiently as possible.

- If advised to conduct a clinical trial, the applicant has to contact the healthcare entities to conduct the trials in accordance with the "Regulation and Requirements for Conducting Clinical Trials on Drug".
- As the sponsor completes the required data, a marketing authorisation application can be submitted through Saudi Drug Registration (SDR) System according to the “Guidance for Submission”.

5. Scientific committee activities

The scientific committee consists of different stakeholder members from SFDA and healthcare entities, and they are conducting the following activities:

- Provide a guidance on an efficient drug development program.
- Perform an assessment for the breakthrough medicine designation application.
- Engaging with medicine developers early on, to improve clinical trial designs that generate robust data on a medicine's benefits and risks and suitable for evaluating a marketing authorisation application.
- Facilitate patient access to breakthrough medicines.

Appendix 1

Breakthrough Medicine Designation Form [name of product]

It is strongly recommended to address all elements outlined below (whenever applicable) with an upper limit of 50 pages.

Please complete this form and send it to SDR.Drug@sfda.gov.sa, titled “Breakthrough Medicine Designation Form [name of product]”

Active Substance(s) name :International Non-proprietary Name (INN):	
Proposed invented name of the medicinal product (Trade name) if known:	
mechanism of action	
Pharmaco-therapeutic group (ATC Code) if known:	
Pharmaceutical form(s) and strength(s):	
Route(s) of administration:	
Proposed indication:	
manufacturing sites of: Drug Substance(s) Drug Product	
clinical stages of development including: clinical trial phase Local clinical trial registration number, if applicable) global clinical trial registration number, if any (e.g. EudraCT, ClinicalTrial.gov)	

Current global regulatory status including: • Application in other global expedited pathways (e.g. PRIME,BTD) • Compassionate/specials usage • Pending and refused marketing authorisation applications • Granted marketing authorisations	
Applicant	

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List of abbreviations

Background information

Background information on the disease
[Outline main features of the disease and current standard therapy, referring to relevant publications. Also, the applicant should include an overview of other developments ongoing in the disease to be treated]

Background information on the product
[Provide a brief description of the product, its mechanism(s) of action and rationale for the use of the medicinal product in the condition]

Development status
[Provide information on the development status of the product on quality, nonclinical and clinical aspects.
Only high level overview is to be presented in this section. Details of the nonclinical and clinical data supporting should be included in section annexes.
A brief summary of planned investigations/studies can also be included here.]

Regulatory status
[Describe the worldwide regulatory strategy for the product's approval, indicating planned type and timelines of marketing authorisation application (MAA).
Indicate whether scientific advice has been previously requested other agencies(e.g. FDA, EMA).
Indicate if the product has been granted or denied support through other development support scheme at national or global level]

Please tick appropriate box:

Medicinal product intended for ☐ diagnosis ☐ prevention ☐ treatment

Of *(name of the condition)*:

_____

Criterion 1

A1. Details of the condition

☐ Life-threatening

☐ Seriously debilitating

Please briefly describe the condition (aetiology, pathophysiology, histopathology, diagnosis and symptomatology) that the medicinal product is intended to diagnose, prevent or treat. The severity of the disease (life-threatening or seriously debilitating) should be justified based on objective and quantifiable medical or epidemiological information in terms of mortality and morbidity with special emphasis on patient quality of life.

A2. Details of the high unmet need

☐ There is no method available.

☐ Existing methods have serious limitations.

Please provide a critical review of the methods of diagnosis, prevention, or treatment used in clinical practice, including an evaluation of the performance and limitations of these methods based on quantifiable data (e.g. data on survival, disease progression/relapses, patient-reported outcomes).

Criterion 2

The medicinal product is likely to offer major advantage over methods currently used.

Please provide a summary of non-clinical and clinical data available to justify the advantage of the product. The Applicant should submit preliminary evidence, based on non-clinical and clinical data, that the advantage claimed for the product may be of significant relevance to the patient and will address their unmet need. A well-argued evaluation of the likelihood of achievement of the product's claim should be provided, based on the totality of information available at the time of designation.

Criterion 3

The potential adverse effects of the medicinal product are likely to be outweighed by the benefits, allowing for the reasonable expectation of a positive benefit/risk balance.

A1. Benefit

Please summarise the overall results of clinical studies and how they translated to the patient's benefit.

A2. Risk

Describe if the clinical development program was halted, amended or had a clinical hold due to safety concerns.
Describe any fatal or life threatening side effects in human subjects (if any).

Please summarise the preliminary scientific evidence showing that the safety profile of the medicinal product is likely to be manageable and acceptable in relation to the estimated benefit.

References

Annexes (supportive non-clinical and clinical data, if required)