

DRAFT SCIENTIFIC OPINION

Scientific and technical guidance for the assessment of products notified as food for special medical purposes in the context of Article 3 of Regulation (EU) No 609/2013¹

EFSA Panel on Dietetic Products, Nutrition and Allergies (NDA)^{2, 3}

European Food Safety Authority (EFSA), Parma, Italy

ABSTRACT

Following a request from the European Commission, the EFSA Panel on Dietetic products, Nutrition and Allergies (NDA) was asked to provide scientific and technical guidance for the assessment of products notified as food for special medical purposes in the context of Article 3 of Regulation (EU) No 609/2013. The guidance presented in this document is for assisting in the preparation and presentation of well-structured dossiers for food products notified as foods for special medical purposes (FSMPs). It presents a common format for the organisation of the information to be provided and outlines the information and scientific data which must be included in the dossier, as well as the key issues which should be addressed in the dossier in order to assess the extent to which a food product notified as FSMP falls under the scope of Regulation (EU) No 609/2013, under the proposed use. It is intended that the guidance will be kept under review and will be further amended and updated as appropriate in the light of experience gained from the evaluation of dossiers for specific food products notified as FSMP, and in the light of future Community guidelines and legislation. The scope of this guidance is limited to FSMPs in the context of Article 3 of Regulation (EU) No 609/2013. Out of the scope of this guidance are: a) other categories of food falling under Regulation (EU) No 609/2013, such as infant formula and follow-on formula, processed cereal-based food and baby food, and total diet replacement for weight control; b) meal replacements for weight control; c) foods “gluten-free” and “lactose-free”.

© European Food Safety Authority, 2015

KEY WORDS

food product, disease, disorder, medical condition, patients, dietary management

¹ On request from the European Commission, Question No EFSA-Q-2014-00736, endorsed on 29 June 2015 for public consultation.

² Panel members: Carlo Agostoni, Roberto Berni Canani, Susan Fairweather-Tait, Marina Heinonen, Hannu Korhonen, Sébastien La Vieille, Rosangela Marchelli, Ambroise Martin, Androniki Naska, Monika Neuhäuser-Berthold, Grażyna Nowicka, Yolanda Sanz, Alfonso Siani, Anders Sjödin, Martin Stern, Sean (J.J.) Strain, Inge Tetens, Daniel Tomé, Dominique Turck and Hans Verhagen. Correspondence: nda@efsa.europa.eu

³ Acknowledgement: The Panel wishes to thank the members of the ad-hoc Working Group on food for special medical purposes: Hildegard Przyrembel, Alfonso Siani, Dominique Turck and André Van Gossum for the preparatory work on this scientific opinion and EFSA staff: Silvia Valtueña Martínez for the support provided to this scientific opinion.

Suggested citation: EFSA NDA Panel (EFSA Panel on Dietetic Products, Nutrition and Allergies), 2015. Draft Scientific and technical guidance for the assessment of products notified as food for special medical purposes in the context of Article 3 of Regulation (EU) No 609/2013. EFSA Journal 2015;volume(issue):NNNN, 23 pp. doi:10.2903/j.efsa.2015.NNNN

Available online: www.efsa.europa.eu/efsajournal

SUMMARY

Following a request from the European Commission, the EFSA Panel on Dietetic products, Nutrition and Allergies (NDA) was asked to provide scientific and technical guidance for the assessment of products notified as food for special medical purposes in the context of Article 3 of Regulation (EU) No 609/2013.

The guidance presented in this document is for assisting in the preparation and presentation of well-structured dossiers for food products notified as foods for special medical purposes (FSMPs). It presents a common format for the organisation of the information to be provided and outlines the information and scientific data which must be included in the dossier, as well as the key issues which should be addressed in the dossier in order to assess the extent to which a food product notified as FSMP falls under the scope of Regulation (EU) No 609/2013, under the proposed use.

It is intended that the guidance will be kept under review and will be further amended and updated as appropriate in the light of experience gained from the evaluation of dossiers for specific food products notified as FSMP, and in the light of future Community guidelines and legislation.

The scope of this guidance is limited to FSMPs in the context of Article 3 of Regulation (EU) No 609/2013. Out of the scope of this guidance are:

- a) other categories of food falling under Regulation (EU) No 609/2013, such as infant formula and follow-on formula, processed cereal-based food and baby food, and total diet replacement for weight control;
- b) meal replacements for weight control;
- c) foods “gluten-free” and “lactose-free”.

The dossier must contain information on:

- a) the characteristics of the food product proposed as FSMP. This information should contain aspects such as the composition, physical and chemical characteristics, manufacturing process, and stability;
- b) the disease/disorder or the medical condition, including the criteria used for the diagnosis of the disease/disorder, or a detailed description of the medical condition;
- c) the characteristics of patients suffering from the disease/disorder or the medical condition for which the food product is intended, including the reasons why such patients would have a nutritional disadvantage from consuming ordinary foodstuffs only, i.e. the dietary management of such patients cannot be achieved or is difficult to achieve by modifications to the normal diet alone, including the addition of food supplements and/or fortified foods;
- d) the proposed conditions of use, including the quantity and pattern of consumption and directions for the preparation and/or use;
- e) the specific role of the food product proposed as FSMP in the dietary management of patients with the disease/disorder or the medical condition for which the food product is intended, including information on how the food product is different from/more suitable than ordinary foodstuffs and evidence that the food product is effective in meeting the nutritional requirements of such patients;
- f) any potential restrictions of use.

On the basis of the data provided in the dossier, the NDA Panel will evaluate the extent to which:

- a) the food product proposed as FSMP is characterised;
- b) the disease/disorder or the medical condition is characterised;

- 73 c) the dietary management of patients with the disease/disorder or the medical condition is
74 not possible or is difficult to achieve by using ordinary foodstuffs or by modifying the
75 normal diet;
- 76 d) the food product is different from / more suitable than ordinary foodstuffs for the dietary
77 management of patients for whom it is intended;
- 78 e) the food product is effective in meeting the nutritional requirements of the patients for
79 whom it is intended under the proposed conditions of use;
- 80 f) restrictions of use apply.

TABLE OF CONTENTS

Abstract	1
Summary	2
Background as provided by the European Commission.....	5
Terms of reference as provided by the European Commission.....	8
Objectives.....	9
Scope.....	9
General principles	9
Organisation and content of the dossier	12
1. Part 1: Administrative and technical data	14
1.1. Comprehensive table of contents of the dossier.....	14
1.2. Identification form	14
1.3. Party responsible for the dossier	14
1.4. Specifications.....	14
1.5. Confidential data.....	15
1.6. References and supporting documentation	15
2. Part 2: Characterisation of the food product proposed as FSMP.....	15
2.1. Name and characteristics	15
2.2. Manufacturing process.....	15
2.3. Stability information	15
2.4. References.....	16
3. Part 3: Characterisation of the disease/disorder or the medical condition for which the food product proposed as FSMP is intended	16
3.1. Diagnosis of the disease/disorder – description of the medical condition	16
3.2. Impact of the disease/disorder or the medical condition on the nutritional status of the patients for whom the food product proposed as FSMP is intended.....	16
3.3. References.....	17
4. Part 4: Characterisation of the patients suffering from the disease/disorder or the medical condition for which the specific food product is intended.....	18
4.1. Patients.....	18
4.2. Nutritional disadvantage	18
4.3. Guidelines for the dietary management of patients.....	20
4.4. References.....	20
5. Part 5: Proposed conditions and restrictions of use	20
5.1. Conditions of use	20
5.2. Restrictions of use.....	21
5.3. References.....	21
6. Part 6: Definition of the specific role of the food product proposed as FSMP in the dietary management of patients with the disease/disorder or medical condition for which the food product is intended.....	21
6.1. Specific characteristics of the food product	21
6.2. Efficacy of the food product in meeting the nutritional requirements of the patients for whom it is intended.....	21
6.3. References.....	21
Glossary and abbreviations	23

BACKGROUND AS PROVIDED BY THE EUROPEAN COMMISSION

Existing legal framework for dietary food for special medical purposes (FSMPs) and its application

Commission Directive 1999/21/EC⁴ lays down composition and labelling requirements for dietary food for special medical purposes (FSMPs) under the framework of Directive 2009/39/EC of the European Parliament and of the Council on foods intended for particular nutritional uses (also called "dietetic foods")⁵.

According to Article 1(2)(b) of Directive 1999/21/EC "*dietary foods for special medical purposes*' means a category of foods for particular nutritional uses specially processed or formulated and intended for the dietary management of patients and to be used under medical supervision. They are intended for the exclusive or partial feeding of patients with a limited, impaired or disturbed capacity to take, digest, absorb, metabolise or excrete ordinary foodstuffs or certain nutrients contained therein or metabolites, or with other medically-determined nutrient requirements, whose dietary management cannot be achieved only by modification of the normal diet, by other foods for particular nutritional uses, or by a combination of the two."⁶

Article 3 of Directive 1999/21/EC sets the requirement that the formulation of FSMPs shall be based on sound medical and nutritional principles. Their use, in accordance with the manufacturer's instructions, shall be safe and beneficial and effective in meeting the particular nutritional requirements of the persons for whom they are intended, as demonstrated by generally accepted scientific data. Certain compositional criteria for FSMPs are set out in the Annex to the Directive. It is however foreseen that FSMPs can derogate from the compositional requirements if these are contrary to the intended use of the product. This flexibility is needed taking into account the variety of conditions for the dietary management of which FSMPs are intended.

Article 4 of Directive 1999/21/EC sets a series of labelling requirements for FSMPs and Article 5 requires operators to notify the placing on the market of FSMPs to national competent authorities (unless Member States consider this notification not necessary).

National competent authorities have reported that application of the current legislative framework applicable to FSMPs is becoming challenging, that it may differ from one Member State to the other and that particular attention has to be paid to the definition of these products. Member States' experts have in particular flagged that an increasing number of products are notified as FSMPs in their territory, very often with doubts arising on whether these products really comply with the definition of FSMP. This situation may be detrimental to the interests of consumers, can affect competition and may pose challenges for the free circulation of goods in the EU.

In this context, the Commission services have taken note of the requests of Member States to develop a guidance document that could assist Member States' competent authorities in their enforcement tasks and stakeholders in marketing their products under the appropriate legal framework. Work on this guidance is currently on-going.

⁴ OJ L 91, 7.4.1999, p. 29

⁵ OJ L 124, 20.5.2009, p. 21

⁶ This definition is similar to that given in the CODEX Standard for the labelling of and claims for foods for special medical purposes (Codex STAN 180-1991). According to Article 1(3) of Directive 1999/21/EC, FSMPs are classified into three categories (a) nutritionally complete foods with a standard nutrient formulation which, used in accordance with the manufacturer's instructions, may constitute the sole source of nourishment for the persons for whom they are intended; (b) nutritionally complete foods with a nutrient-adapted formulation specific for a disease, disorder or medical condition which, used in accordance with the manufacturer's instructions, may constitute the sole source of nourishment for the persons for whom they are intended; (c) nutritionally incomplete foods with a standard formulation or a nutrient-adapted formulation specific for a disease, disorder or medical condition which are not suitable to be used as the sole source of nourishment. The foods referred to in points (a) and (b) may also be used as a partial replacement or as a supplement to the patient's diet.

The new Framework applicable to FSMPs

Regulation (EU) No 609/2013 of the European Parliament and of the Council on food intended for infants and young children, food for special medical purposes and total diet replacement for weight control⁷ (the FSG Regulation) revises the framework applicable to dietetic foods in general and to FSMPs in particular. The Regulation abolishes the concept of dietetic food, includes FSMPs under its scope, maintains the definition of Directive 1999/21/EC with minor adaptations⁸ and requires the Commission to establish specific composition and information requirements for FSMPs in a delegated act to be adopted by 20 July 2015. The Commission is currently preparing the delegated act that will maintain the existing rules with adaptations where needed.

In order to ensure uniform implementation of the rules, Article 3 of the FSG Regulation empowers the Commission to decide as of 20 July 2016 (date of entry into application of the Regulation) by means of implementing acts:

(a) whether a given food falls within the scope of the Regulation and/or

(b) to which specific category of food covered by the Regulation a given food belongs.

Taking into account that the FSG Regulation does not further specify the procedure to be followed when applying Article 3, the Commission services have started to reflect, together with Member States, on the practical implications of the future application of this Article.

Application of Article 3 of the FSG Regulation for products notified as FSMPs

Given the abovementioned difficulties related to the application of the current FSMPs legislative framework, in particular with respect to the product definition (which has been maintained in the FSG Regulation), it appears evident that Article 3 of the FSG Regulation will be of particular relevance for these products in the future.

In this context, the Commission considers including in the guidance document for FSMPs it is currently working on clear information on how Article 3 of the FSG Regulation could be applied in the case of products notified at national level as FSMPs. The inclusion of this information in the FSMPs guidance would guarantee transparency both for national competent authorities and stakeholders. In addition, this information could constitute useful advice to national competent authorities when applying the relevant rules on a case-by-case basis to products notified as FSMPs (even before it is decided to adopt a decision under Article 3). Finally, the inclusion of this information in the guidance would help operators market their products in accordance with the appropriate rules.

EFSA's role when Article 3 of the FSG Regulation is applied for products notified as FSMPs

Article 7 of the FSG Regulation foresees the possibility to consult EFSA on any matter related to the application of the Regulation which is likely to have an effect on public health. Clearly, EFSA's advice will be crucial for the Commission after 20 July 2016 when preparing implementing decisions pursuant to Article 3 in the case of products notified as FSMPs.

⁷ OJ L 181, 29.6.2013, p. 35

⁸ Article 2(2)(g): "Food for special medical purposes' means food specially processed or formulated and intended for the dietary management of patients, including infants, to be used under medical supervision; it is intended for the exclusive or partial feeding of patients with a limited, impaired or disturbed capacity to take, digest, absorb, metabolise or excrete ordinary food or certain nutrients contained therein, or metabolites, or with other medically-determined nutrient requirements, whose dietary management cannot be achieved by modification of the normal diet alone".

When deciding to adopt an implementing decision on a specific product notified as FSMP, the main point on which the Commission could need EFSA's advice is expected to be the relationship between the product and the disease/disorder/condition for the dietary management of which the product is intended. Indeed, according to the definitions given in Directive 1999/21/EC and the FSG Regulation, FSMPs are intended for the exclusive or partial feeding of patients⁹:

- (a) with a limited, impaired or disturbed capacity to take, digest, absorb, metabolise or excrete ordinary foodstuffs or certain nutrients contained therein or metabolites;
- (b) or with other medically-determined nutrient requirements, whose dietary management cannot be achieved only by modification of the normal diet, by other foods for particular nutritional uses, or by a combination of the two¹⁰.

The main function of FSMPs is therefore to feed patients that, because of a particular disease/disorder/condition, either have problems in consuming ordinary foodstuffs, or have specific medically-determined nutrient requirements whose dietary management cannot be achieved by modifying the normal diet. In both the sub-paragraphs of the definition quoted above, there is a clear indication of the difference between FSMPs and other foodstuffs that are not FSMPs: FSMPs are foods whose consumption by patients is necessary because it is impossible, very difficult or unrealistic for these patients to satisfy their nutritional needs through the consumption of foods other than FSMPs¹¹.

This is why, after 20 July 2016 (when Article 3 of the FSG Regulation becomes applicable), EFSA might be requested to provide advice on a specific product notified as FSMP and to inform the Commission whether it is recognised, on the basis of generally accepted scientific data, that people suffering from the specific disease/disorder/condition for the dietary management of which the product is intended:

- (a) are in the impossibility or difficulty to take, digest, absorb, metabolise or excrete ordinary foodstuffs, or certain nutrients contained therein or metabolites or
- (b) have specific medically-determined nutrient requirements, typical to the disease/disorder/condition, that cannot be reasonably or realistically satisfied by modifying the normal diet.

EFSA might be requested to inform the Commission about the degree to which it would be impossible or difficult to consume ordinary foodstuffs and/or to what extent a modification of the normal diet would not reasonably or realistically cater for the specific nutrient requirements of the patient in the specific case. In this context, EFSA might be requested to advise the Commission on the role of the specific product, namely whether and how is the specific product different from/more suitable than foods that are not FSMPs, taking into account its composition, its intended use and the proposed instructions for use (including patterns of consumption).

Requests for EFSA's advice to inform the preparation of Commission's implementing decisions pursuant to Article 3 of the FSG Regulation for products notified as FSMPs will not be sent to EFSA before 20 July 2016.

⁹ For the purposes of this exercise, the concept of 'patient' has to be interpreted in a broad way, as people suffering from a diagnosed disease, disorder or medical condition.

¹⁰ Reference to "*foods for particular nutritional uses*" has disappeared in the definition given in the FSG Regulation.

¹¹ Food supplements or fortified foods are regulated as foods and should therefore, for the purpose of this exercise, be considered as "ordinary foodstuffs" (see first bullet point quoting the definition above). Similarly, a "modification of the normal diet" (see second bullet point quoting the definition above) should be considered as any adjustment to the diet through consumption of foods other than FSMPs and can include use of food supplements or fortified foods.

However, in preparation for future work, the Commission considers it necessary to consult EFSA at this stage regarding the type of data that food business operators should make available to EFSA in the future in order for EFSA to provide scientific advice to the Commission along the lines described above.

This advice from EFSA could eventually also be included in the guidance document currently being drafted by the Commission on FSMPs so that the document would describe procedural aspects of the application of the Article 3 procedure (e.g. details about how to request the Commission's intervention on a specific product) as well as information on how to prepare a dossier for EFSA's assessment.

TERMS OF REFERENCE AS PROVIDED BY THE EUROPEAN COMMISSION

In accordance with Article 29 of Regulation (EC) No 178/2002, the European Commission requests the European Food Safety Authority to issue an opinion on scientific and technical guidance regarding the type of data that will be needed by EFSA for providing scientific advice to the Commission, after 20 July 2016, on specific products notified as FSMPs at national level and for which the Commission intends to adopt implementing decisions pursuant to Article 3 of Regulation (EU) No 609/2013.

In preparing this scientific and technical guidance, EFSA is requested to take into account the type of scientific advice that the Commission will need when taking decisions pursuant to Article 3 of Regulation (EU) No 609/2013. In this context, after 20 July 2016, EFSA might for example be asked to advise on:

1. the extent to which the disease/disorder/condition for which the specific product is intended is sufficiently characterised;
2. the extent to which patients suffering from the specific disease/disorder/condition for the dietary management of which the product is intended:
 - a. are in the impossibility or difficulty to take, digest, absorb, metabolise or excrete ordinary foodstuffs, or certain nutrients contained therein or metabolites or
 - b. have specific medically-determined nutrient requirements, typical to the disease/disorder/condition, that cannot be reasonably or realistically satisfied by modifying the normal diet;
3. the extent to which the specific product is sufficiently characterised;
4. the specific role of the product in the dietary management of the disease/disorder/condition for which it is intended, in particular the extent to which the specific product is different from / more suitable than foods that are not FSMPs, taking into account its composition, its intended use and the proposed instructions of use (including patterns of consumption);
5. any potential restrictions of use.

OBJECTIVES

The guidance presented in this document is for assisting in the preparation and presentation of well-structured dossiers for food products notified as foods for special medical purposes (FSMPs) in the context of Article 3 of Regulation (EU) No 609/2013.

It presents a common format for the organisation of the information to be provided and outlines:

- the information and scientific data which must be included in the dossier,
- the key issues which should be addressed in the dossier in order to assess the extent to which a food product notified as FSMP falls under the scope of Regulation (EU) No 609/2013, under the proposed use.

It is intended that the guidance will be kept under review and will be further amended and updated as appropriate in the light of experience gained from the evaluation of dossiers for specific food products notified as FSMP, and in the light of future Community guidelines and legislation.

SCOPE

The scope of this guidance is limited to FSMPs in the context of Article 3 of Regulation (EU) No 609/2013.

Out of the scope of this guidance are:

- a) other categories of food falling under Regulation (EU) No 609/2013, such as infant formula and follow-on formula, processed cereal-based food and baby food, and total diet replacement for weight control;
- b) meal replacements for weight control;
- c) foods “gluten-free” and “lactose-free”.

GENERAL PRINCIPLES

This document should be read in conjunction with Regulation (EU) No 609/2013 of the European Parliament and of the Council as regards FSMP, with Directive 2002/46/EC of the European Parliament and of the Council on the approximation of the laws of the Member States relating to food supplements, as amended¹², Regulation (EC) No 1925/2006 of the European Parliament and of the Council on the addition of vitamins and minerals and of certain other substances to foods¹³, as amended, and with current and future Community guidelines and Regulations.

1. This guidance applies to FSMP as described in Regulation (EU) No 609/2013, i.e. “*food specially processed or formulated and intended for the dietary management of patients, including infants, to be used under medical supervision; it is intended for the exclusive or partial feeding of patients with a limited, impaired or disturbed capacity to take, digest, absorb, metabolise or excrete ordinary food or certain nutrients contained therein, or metabolites, or with other medically-determined nutrient requirements, whose dietary management cannot be achieved by modification of the normal diet alone*”.

2. In the context of this guidance:

Disease/disorder means a pathological process, acute or chronic, inherited or acquired, of known or unknown origin, having a characteristic set of signs and symptoms which are used for its diagnosis and the management of which requires nutritional intervention under medical

¹² OJ L 183, 12.7.2002, p. 51

¹³ OJ L 404, 30.12.2006, p. 26

supervision. In this guidance document, the terms disease and disorder are considered as synonymous and have the same meaning.

Medical condition denotes any structural or functional alteration, either acute or chronic, which may result from one or more diseases or disorders, the management of which requires nutritional intervention under medical supervision¹⁴.

Patient refers to a person (including infants and young children) affected by the **disease/disorder or the medical condition**.

Food product denotes any food suitable for human consumption¹⁵ (i.e. to be administered by the oral or enteral route) which provides energy-containing macronutrients (e.g. carbohydrates, protein, fats), and/or micronutrients (e.g. vitamins, minerals), and/or other substances which may contribute to fulfil the nutritional requirements of **patients**.

Food supplements means foodstuffs the purpose of which is to supplement the normal diet and which are concentrated sources of nutrients (i.e. vitamins, minerals) or other substances with a nutritional or physiological effect, alone or in combination, marketed in dose form, namely forms such as capsules, pastilles, tablets, pills and other similar forms, sachets of powder, ampoules of liquids, drop dispensing bottles, and other similar forms of liquids and powders designed to be taken in measured small unit quantities¹⁶.

Fortified food means a foodstuff to which vitamin(s), mineral(s), other substances, or a combination of them, have been added¹⁷.

Ordinary foodstuff means any food available in the market intended for human consumption which may be part of the normal diet, or may be used to supplement the normal diet, of subjects belonging to the general population or population subgroups thereof. In this guidance, **fortified foods** and **food supplements** are considered **ordinary foodstuffs**.

Modifications of the normal diet means changes introduced into a subject's habitual diet by using ordinary foodstuffs only.

Proposed use denotes the context in which the food product is proposed as FSMP, including the target **patient** population, the **disease/disorder or the medical condition**, the conditions of use (i.e. quantity and pattern of consumption, directions for preparation and use), and, where applicable, the restrictions of use.

3. The term **dossier** hereafter means a stand-alone package containing the information and scientific data submitted for the scientific evaluation of a food product notified as FSMP under the proposed use.
4. One dossier should be prepared for each individual food product and proposed use; this means that only **a food product and a single disease/disorder OR a single medical condition can be the object of each dossier**. However, multiple versions¹⁸ of the food product can be

¹⁴ Examples (non-exhaustive list) of medical conditions resulting from different diseases/disorders are, e.g.: a) liver failure resulting from e.g. viral hepatitis, hemochromatosis, Wilson's disease; b) dysphagia resulting from e.g. cancer of the upper gastrointestinal tract, neurological disorders (e.g. multiple sclerosis, muscular dystrophy, Parkinson's disease); c) respiratory failure resulting from e.g. cystic fibrosis, Duchenne myopathy, α 1-antitrypsin deficiency; d) short bowel syndrome resulting from e.g. inflammatory bowel disease, necrotizing enterocolitis; e) chronic metabolic acidosis resulting from e.g. inherited renal tubular acidosis, organic acidaemias (e.g. maple syrup urine disease, propionic acidaemia).

¹⁵ Products intended for administration via other routes (e.g. intravenous, intramuscular, cutaneous, by inhalation) are out of the scope of this guidance

¹⁶ Adapted from Directive 2002/46/EC

¹⁷ As laid down in Commission Regulation (EC) No 1925/2006, as amended

¹⁸ For example, a food product available in different flavours (e.g. vanilla, chocolate) or formats (tablets, powder, liquid).

proposed as FSMP in the same dossier, provided that the scientific evidence is valid for all proposed versions of the food product.

5. The dossier should contain all pertinent information and scientific data (published and unpublished, data in favour and not in favour) relative to the assessment of the food product as FSMP under the proposed use.

6. The dossier in itself cannot be confidential. Sections considered as confidential by the party responsible for the dossier should be kept to a minimum. In addition, EFSA requests the party responsible for the dossier to identify and justify those parts of the dossier claimed as confidential. For example, confidentiality can only be given to specific parts of a study if duly justified, and not to an entire study. However, in principle and without prejudice to Regulation (EC) No 1049/2001 on public access to documents, if a study has not yet been published and its disclosure would undermine the commercial interests and rights of the party responsible for the dossier, EFSA will not make such study available to third parties. In order to comply with its requirements for transparency as outlined in Article 38 of Regulation (EC) No 178/2002¹⁹, EFSA needs to disclose in its scientific opinions key data from dossiers which are considered essential for the scientific assessment. If the request for confidential treatment for those parts identified by the party responsible for the dossier is accompanied by verifiable justification and if this is accepted by EFSA, those elements will be kept confidential in the final scientific opinion.

7. This guidance presents a common format for the organisation of the information. Adherence to this format will also facilitate access to the information by the NDA Panel in order to conduct the evaluation and deliver scientific advice in an effective and consistent way.

8. Not all the information and data specified in this guidance will be required for each dossier. However, reasons/justification must be given for the absence of such data in the dossier.

9. The dossier must contain information on:

- a) the characteristics of the food product proposed as FSMP. This information should contain aspects such as the composition (e.g. ingredients and their sources, concentrations of macro- and micronutrients), physical and chemical characteristics, manufacturing process, and stability;
- b) the disease/disorder or the medical condition, including the criteria used for the diagnosis of the disease/disorder, or a detailed description of the medical condition;
- c) the characteristics of patients suffering from the disease/disorder or the medical condition for which the food product is intended, including the reasons why such patients would have a nutritional disadvantage from consuming ordinary foodstuffs only, i.e. why the dietary management of such patients cannot be achieved or is difficult to achieve by modifications to the normal diet alone, including the addition of food supplements and/or fortified foods²⁰;
- d) the proposed conditions of use, including the quantity and pattern of consumption and directions for the preparation and/or use;

¹⁹ Regulation (EC) No 178/2002 of the European Parliament and of the Council of 28 January 2002 laying down the general principles and requirements of food law, establishing the European Food Safety Authority and laying down procedures in matters of food safety, as last amended.

²⁰ For example, the nutritional requirements of patients needing tube feeding could be met by mixing, homogenising and diluting normal foods. However, the process is time consuming, technically complex and potentially harmful (e.g. infectious complications), so that the use of ready-to-feed products for enteral nutrition could be justified for the dietary management of these patients.

e) the specific role of the food product proposed as FSMP in the dietary management of patients with the disease/disorder or the medical condition for which the food product is intended, including information on how the food product is different from ordinary foodstuffs and evidence that the food product is effective in meeting the nutritional requirements of such patients;

f) any potential restrictions of use.

10. On the basis of the data provided in the dossier, the NDA Panel will evaluate the extent to which:

a) the food product proposed as FSMP is characterised;

b) the disease/disorder or the medical condition is characterised;

c) the dietary management of patients with the disease/disorder or the medical condition is not possible or is difficult to achieve by using ordinary foodstuffs or by modifications of the normal diet;

d) the food product is different from / more suitable than ordinary foodstuffs for the dietary management of patients for whom it is intended;

e) the food product is effective in meeting the nutritional requirements of the patients for whom it is intended under the proposed conditions of use;

f) restrictions of use apply.

ORGANISATION AND CONTENT OF THE DOSSIER

The following information should be provided in the dossier, and the structure should follow a common format, i.e. order and numbering system of different parts, headings and sub-headings. Data provided in the dossier should be organised into **six Parts**.

Part 1 contains administrative and technical data, such as the identification form, information related to the party responsible for the dossier, and the dossier specifications, including conditions and restrictions of use.

Part 2 contains information relative to the characterisation of the food product proposed as FSMP, including its name and characteristics, list of ingredients, a quantitative analysis of its energy and nutrient content, a description of the manufacturing process, and stability information.

Part 3 contains information relative to the characterisation of the disease/disorder or the medical condition for which the food product proposed as FSMP is intended, including criteria for diagnosis of the disease/disorder or a description of the medical condition, and the impact of the disease/disorder or the medical condition on the nutritional status of patients, if any.

Part 4 comprises information relative to the characterisation of the patients suffering from the disease/disorder or the medical condition for whom the specific food product is intended and to the reason(s) why such patients would have a nutritional disadvantage from consuming ordinary foodstuffs only.

Part 5 contains a description of the proposed conditions of use, including the quantity and pattern of consumption and directions for the preparation and/or use, and a description of any potential restrictions of use.

Part 6 comprises information relative to the specific role of the food product proposed as FSMP in the dietary management of patients with the disease/disorder or the medical condition for which the food product is intended, including data on the effectiveness of the food product in meeting the nutritional requirements of the patients for whom it is intended.

429 Where some of the data that are required as described in this guidance document do not apply to a
430 particular food product, reasons/justification must be given for the absence of such data in the dossier.

1. Part 1: Administrative and technical data

1.1. Comprehensive table of contents of the dossier

1.2. Identification form

Please use the identification form provided in the **Appendix**.

1.3. Party responsible for the dossier

1.3.1. Company/organisation

Provide the name and address of the company or organisation²¹.

1.3.2. Contact person

Indicate the contact person authorised to communicate with EFSA on behalf of the party responsible for the dossier²².

1.4. Specifications

1.4.1. Specify the food product proposed as FSMP

1.4.2. Specify whether the dossier refers to:

☐ A *nutritionally complete* food product with a *standard nutrient* formulation which, used in accordance with the manufacturer's instructions, may constitute the *sole source of nourishment* for the persons for whom it is intended.

☐ A *nutritionally complete* food product with a *nutrient-adapted* formulation specific for a disease, disorder or medical condition which, used in accordance with the manufacturer's instructions, may constitute the *sole source of nourishment* for the persons for whom it is intended.

☐ A *nutritionally incomplete* food product with a standard formulation or a nutrient-adapted formulation specific for a disease, disorder or medical condition which are *not suitable* to be used *as the sole source of nourishment*.

1.4.3. Specify the disease/disorder or the medical condition for which the food product proposed as FSMP is intended

1.4.4. Describe the type of patients for whom the food product proposed as FSMP is intended

1.4.5. Conditions of use

Indicate the quantity of the food product proposed as FSMP and the pattern of consumption and/or mode of administration which would be required for the dietary management of patients for whom the food product is intended.

²¹ In case more than one company or organisation submits dossier, provide their names and addresses. EFSA requires that **only one contact person be authorised to communicate with EFSA**.

²² To facilitate communication, EFSA requires that there be only **one contact person per dossier**.

465

466 **1.4.6. Restrictions of use**

467 Specify the category or categories of patients who should avoid using the food product proposed as
468 FSMP and provide a rationale for the proposed restrictions of use.

469

470 **1.5. Confidential data**

471 State whether the dossier includes confidential data ☐ yes ☐ no

472 If yes, please specify the related Part in the dossier, stating section and page number, and providing (a)
473 verifiable justification(s)/declaration(s) (see also general principle 6 of this guidance document).

474 **1.6. References and supporting documentation**

475 References and supporting documentation quoted under **Part 1** should be given here (no particular
476 format required), together with copies/reprints of published data and/or full reports of unpublished
477 data.

478

479 **2. Part 2: Characterisation of the food product proposed as FSMP**

480 **2.1. Name and characteristics**

481 The source and specifications (e.g. physical and chemical properties, composition, and where
482 applicable microbiological constituents) of the food product proposed as FSMP should be provided
483 and should include the list of ingredients and their sources, as well as a quantitative analysis of the
484 energy and nutrient content of the product as consumed.

485 Analytical methods applied should be scientifically sound and standardised to ensure quality and
486 consistency of the data.

487 Measurements should be performed in a competent laboratory that can certify the data. Whenever a
488 quality system is in place for control/documentation (e.g. GLP and ISO17025) the particular system
489 should be indicated.

490

491 **2.2. Manufacturing process**

492 A description of the manufacturing process should be provided. If the production follows a quality
493 system (e.g. GMP) the particular system should be indicated. The variability from batch to batch
494 should be addressed.

495

496 **2.3. Stability information**

497 Where applicable, a brief summary of the studies undertaken (e.g. conditions, batches and analytical
498 procedures), and of the results and conclusions of the stability studies, should be provided.
499 Conclusions with respect to storage conditions and shelf-life should be given.

500

2.4. References

References and supporting documentation quoted under Part 2 should be given here (no particular format required), together with copies/reprints of published data and/or full reports of unpublished data.

3. Part 3: Characterisation of the disease/disorder or the medical condition for which the food product proposed as FSMP is intended

3.1. Diagnosis of the disease/disorder – description of the medical condition

For the disease/disorder or the medical condition for which the food product proposed as FSMP is intended, please specify whether:

- a) It is a disease or disorder²³, the diagnosis of which relies on widely accepted, well-defined, objective criteria (i.e. the criteria used for diagnosis are widely accepted by the medical community and can be verified by a physician)

☐ yes ☐ no

If yes, please specify which are the criteria used for diagnosis. Guidelines/consensus papers published by scientific (medical) societies in which the criteria used for diagnosis are described should also be provided, whenever available.

- b) It is a medical condition²⁴

☐ yes ☐ no

If yes, please specify and describe in detail the medical condition.

3.2. Impact of the disease/disorder or the medical condition on the nutritional status of the patients for whom the food product proposed as FSMP is intended

The disease/disorder or the medical condition has an impact on the nutritional status of the patients for whom the specific food product is intended:

☐ yes ☐ no

If yes, please specify whether the disease/disorder or the medical condition leads to:

- a) Energy/protein malnutrition

☐ yes ☐ no

If yes, please provide evidence that the disease/disorder or the medical condition leads to energy/protein malnutrition

- b) Excess/deficiency of essential amino acids

☐ yes ☐ no

²³ As defined in general principle 2 of this guidance document

²⁴ As defined in general principle 2 of this guidance document

534 If yes, provide evidence that the disease/disorder or the medical condition leads to
 535 deficiency or excess of (essential) amino acid(s) and specify which amino acids are
 536 affected

537

538 c) Deficiency of essential fatty acids

539 ☐ yes ☐ no

540 If yes, provide evidence that the disease/disorder or the medical condition leads to
 541 essential fatty acid deficiency and specify which essential fatty acid(s) are affected

542

543 d) Vitamin deficiency

544 ☐ yes ☐ no

545 If yes, please provide evidence that the disease/disorder or the medical condition leads to
 546 vitamin deficiency and specify which vitamins are affected.

547

548 e) Deficiency of minerals

549 ☐ yes ☐ no

550 If yes, please provide evidence that the disease/disorder or the medical condition leads to
 551 mineral deficiency and specify which essential minerals are affected.

552

553 f) Other impacts on nutritional status

554 ☐ yes ☐ no

555 If yes, please specify

556

557 The potential impact of inflammation resulting from some diseases/disorders or medical conditions on
 558 nutrient biomarker use and interpretation should be considered and discussed, where applicable.

559 3.3. References

560 References and supporting documentation quoted under **Part 3** should be given here (no particular
 561 format required), together with copies/reprints of published data and/or full reports of unpublished
 562 data.

563

4. Part 4: Characterisation of the patients suffering from the disease/disorder or the medical condition for which the specific food product is intended

4.1. Patients

Please specify the patients suffering from the disease/disorder or the medical condition for which the specific food product is intended. Information should be provided on whether the specific food product is intended for all patients suffering from the disease/disorder or the medical condition. If the specific food product is intended only for a particular subgroup of patients suffering from the disease/disorder or the medical condition, please specify the characteristics of such patients (e.g. age, sex, stage of the disease, clinical condition).

4.2. Nutritional disadvantage

Specify the reason(s) why such patients should have a nutritional disadvantage from consuming ordinary foodstuffs only²⁵:

a) Inability (or reduced ability) to *chew and/or swallow* ordinary foodstuffs

☐ yes ☐ no

If yes, please specify the reason why the disease/disorder or the medical condition for which the food product proposed as FSMP is intended would lead to the inability (or reduced ability) to chew and/or swallow ordinary foodstuffs.

b) Inability (or reduced ability) to *digest* ordinary foodstuffs

☐ yes ☐ no

If yes, please specify the reason why the disease/disorder or the medical condition for which the food product proposed as FSMP is intended would lead to the inability (or reduced ability) to digest ordinary foodstuffs.

c) Inability (or reduced ability) to *absorb* nutrients contained in ordinary foodstuffs

☐ yes ☐ no

If yes, please specify the reason why the disease/disorder or the medical condition for which the food product proposed as FSMP is intended would lead to the inability (or reduced ability) to absorb nutrients (please specify which nutrients) contained in ordinary foodstuffs.

d) Inability (or reduced ability) to *metabolise and/or utilise* nutrients contained in ordinary foodstuffs

☐ yes ☐ no

²⁵ For the purpose of this guidance, food supplements and fortified foods are considered ordinary foodstuffs.

599 If yes, please specify the reason why the disease/disorder or the medical condition for
 600 which the food product proposed as FSMP is intended would lead to the inability (or
 601 reduced ability) to metabolise and/or utilise nutrients (please specify which nutrients)
 602 contained in ordinary foodstuffs.

603
 604 e) Inability (or reduced ability) to **excrete** nutrients contained in ordinary foodstuffs, and/or
 605 their metabolites

606 ☐ yes ☐ no

607 If yes, please specify the reason why the disease/disorder or the medical condition for
 608 which the food product proposed as FSMP is intended would lead to the inability (or
 609 reduced ability) to excrete nutrients (please specify which nutrients) contained in
 610 ordinary foodstuffs, and/or their metabolites (please specify which metabolites).

611
 612 f) The disease/disorder or the medical condition is triggered or aggravated by the
 613 consumption of ordinary foodstuffs²⁶.

614 ☐ yes ☐ no

615 If yes, please provide evidence that the disease/disorder or the medical condition is
 616 triggered or aggravated by the consumption of ordinary foodstuff(s) and specify the
 617 foodstuff(s) which trigger(s) or aggravate(s) the disease/disorder or the medical condition,
 618 and why.

619
 620 g) The disease/disorder or the medical condition leads to specific dietary requirements,
 621 which cannot be fulfilled/are difficult to fulfil by the consumption of ordinary foodstuffs
 622 or by modifications of the normal diet.

623 ☐ yes ☐ no

624 If yes, please specify whether the disease/disorder or the medical condition leads to
 625 specific dietary requirements of:

626 ☐ Energy ☐ Carbohydrates ☐ Fat/fatty acids

627 ☐ Protein/amino acids ☐ Vitamins ☐ Minerals

628 ☐ Other substances. Please specify:

629 Please provide evidence that the disease/disorder or the medical condition leads to specific
 630 dietary requirements, identify the nutrients/other substances for which the dietary
 631 requirements are specific, and provide evidence/a rationale for the reason(s) why such specific
 632 requirements cannot be fulfilled/are difficult to fulfil by the consumption of ordinary
 633 foodstuffs or by modifications of the normal diet.

634
²⁶ For example, consumption of protein-containing ordinary foodstuffs triggers sign(s)/symptom(s) of phenylketonuria due to the reduced ability of the body to metabolise the amino acid phenylalanine.

h) Other reasons

☐ yes ☐ no

If yes, please specify

4.3. Guidelines for the dietary management of patients

Please provide guidelines/consensus papers published by scientific (medical) societies for the dietary management of patients with the disease/disorder or the medical condition for which the food product proposed as FSMP is intended, if available.

4.4. References

References and supporting documentation quoted under Part 4 should be given here (no particular format required), together with copies/reprints of published data and/or full reports of unpublished data.

5. Part 5: Proposed conditions and restrictions of use

5.1. Conditions of use

a) Indicate the quantity of the food product proposed as FSMP and the pattern of consumption required for the dietary management of patients for which the food product is intended.

b) The food product is intended to be used as:

- ☐ the sole source of nourishment
- ☐ a partial replacement of other dietary sources
- ☐ a supplement/integration to the patient's diet

c) Route of administration. The food product is intended for:

- ☐ oral nutrition
- ☐ tube feeding: in the stomach ☐
- in the jejunum ☐

d) Specify, where applicable, directions for preparation and/or use.

e) Specify, where applicable, the reasons why the use of the food product requires medical supervision (e.g. tube feeding, adverse effects, control of clinical and/or laboratory outcomes)

5.2. Restrictions of use

- a) Provide, where appropriate, a statement addressed to those patients who should avoid using the food product proposed as FSMP, and include the rationale.

- b) Specify, where applicable, other restrictions of use, and provide a rationale.

5.3. References

References and supporting documentation quoted under **Part 5** should be given here (no particular format required), together with copies/reprints of published data and/or full reports of unpublished data.

6. Part 6: Definition of the specific role of the food product proposed as FSMP in the dietary management of patients with the disease/disorder or medical condition for which the food product is intended

6.1. Specific characteristics of the food product

- a) Please describe the rationale for the specific composition of the food product proposed as FSMP in relation to the pathophysiology of the disease/disorder or medical condition for which the FSMP is intended.

- b) Please specify why the food product proposed as FSMP is different from / more suitable than ordinary foodstuffs for the dietary management of patients for whom it is intended, taking into account its composition and conditions of use (including the directions for preparation and/or use, as well as the proposed quantity and pattern of consumption). Please clarify why the dietary management of such patients cannot be achieved/is difficult to achieve by modifying the normal diet, including the use of food supplements and/or fortified foods, and provide evidence/a rationale.

6.2. Efficacy of the food product in meeting the nutritional requirements of the patients for whom it is intended

Please provide human studies documenting the use of the food product for the dietary management of patients for whom it is intended. If the food is intended for more than one subgroup of patients defined on the basis of the stage of the disease²⁷, evidence/a rationale should be provided for each patient subgroup. Please describe the impact of the food product on the clinical condition of the patients.

6.3. References

References and supporting documentation quoted under **Part 6** should be given here (no particular format required), together with copies/reprints of published data and/or full reports of unpublished data.

²⁷ For example, patients with Crohn's disease during a flare-up and patients with Crohn's disease in remission.

Appendix A. Identification form

IDENTIFICATION FORM



The identification form should be used for a dossier on a food product notified as food for special medical purposes (FSMP) to an EU Member State, and for scientific evaluation by the European Food Safety Authority (EFSA) in the context of Article 3 of Regulation (EU) No 609/2013.

A separate dossier for each food product notified as FSMP is required.

DECLARATION and SIGNATURE

Food product notified as FSMP:

Proposed use²⁸:

Party responsible for the dossier (Company) name:

Address:

Country:

Contact person's name:

Address:

Country:

Telephone:

Fax:

e-mail:

It is hereby confirmed, to our best knowledge, that all existing data which are relevant to the dossier have been supplied, as appropriate.

On behalf of the applicant:

Signature

Name

Function

Place and date (yyyy-mm-dd)

²⁸ It refers to the context in which the food product is proposed as FSMP, including the target patient population, the disease/disorder or the medical condition, the conditions of use (i.e. quantity and pattern of consumption, directions for preparation and use), and, where applicable, the restrictions of use.

742 **GLOSSARY AND ABBREVIATIONS**

Disease/disorder	a pathological process, acute or chronic, inherited or acquired, of known or unknown origin, having a characteristic set of signs and symptoms which are used for its diagnosis and the management of which requires nutritional intervention under medical supervision
Dossier	a stand-alone package containing the information and scientific data submitted for the scientific evaluation of a food product notified as FSMP under the proposed use
Food product	any food suitable for human consumption (i.e. to be administered by the oral or enteral route) which provides energy-containing macronutrients (e.g. carbohydrates, protein, fats), micronutrients (e.g. vitamins, minerals), and/or other substances which may contribute to fulfil the nutritional requirements of patients
Food supplements	foodstuffs the purpose of which is to supplement the normal diet and which are concentrated sources of nutrients (i.e. vitamins, minerals) or other substances with a nutritional or physiological effect, alone or in combination, marketed in dose form, namely forms such as capsules, pastilles, tablets, pills and other similar forms, sachets of powder, ampoules of liquids, drop dispensing bottles, and other similar forms of liquids and powders designed to be taken in measured small unit quantities
Fortified food	a foodstuff to which vitamin(s), mineral(s), other substances, or a combination of them, have been added
FSG Regulation	Regulation (EU) No 609/2013 of the European Parliament and of the Council on food intended for infants and young children, food for special medical purposes and total diet replacement for weight control
Medical condition	any structural or functional alteration, either acute or chronic, which may result from one or more diseases or disorders, the management of which requires nutritional intervention under medical supervision
Modifications of the normal diet	changes introduced into a subject's habitual diet by using ordinary foodstuffs only
Patient	a person (including infants and young children) affected by the disease/disorder or the medical condition
Proposed use	context in which the food product is proposed as FSMP, including the target patient population, the disease/disorder or the medical condition, the conditions of use (i.e. quantity and pattern of consumption, directions for preparation and use), and, where applicable, the restrictions of use