20 June 2025
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EMADOC-1700519818-2253874
Committee for Orphan Medicinal Products

Orphan Maintenance Assessment Report

of an orphan medicinal product submitted for marketing authorisation application

Ogsiveo (nirogacestat)
Treatment of soft tissue sarcoma
EU/3/19/2214

Sponsor: Springworks Therapeutics Ireland Limited

Note

Assessment report as adopted by the COMP with all information of a commercially confidential nature deleted.

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1. Product and administrative information

Product	
Designated active substance(s)	Nirogacestat
Other name(s)	Nirogacestat hydrobromide; PF 3084014; PF-03084014; PF-03084014-04
International Non-Proprietary Name	Nirogacestat
Tradename	Ogsiveo
Orphan condition	Treatment of soft tissue sarcoma
Sponsor's details:	Springworks Therapeutics Ireland Limited Hamilton House 28 Fitzwilliam Place Dublin 2 Co. Dublin D02 P283 Ireland
Orphan medicinal product designation procedural history	
Sponsor/applicant	Voisin Consulting S.A.R.L.
COMP opinion	12 September 2019
EC decision	17 October 2019
EC registration number	EU/3/19/2214
Post-designation procedural history	
Sponsor's name change	Name change from Voisin Consulting S.A.R.L.to Voisin Consulting Life Sciences– EC letter of 12 October 2021
Transfer of sponsorship	Transfer from Voisin Consulting Life Sciences to Springworks Therapeutics Ireland Limited – EC decision of 29 November 2023
Marketing authorisation procedural history	
Rapporteur / Co-rapporteur	Filip Josephson / Margareta Bego
Applicant	Springworks Therapeutics Ireland Limited
Application submission	9 February 2024
Procedure start	29 February 2024
Procedure number	EMA/H/C/006071
Invented name	Ogsiveo
Therapeutic indication	Ogsiveo as monotherapy is indicated for the treatment of adult patients with progressing desmoid tumours who require systemic treatment. Further information on Ogsiveo can be found in the European public assessment report (EPAR) on the Agency's website https://www.ema.europa.eu/en/medicines/human/EPAR/ogsiveo
CHMP opinion	19 June 2025

COMP review of orphan medicinal product designation procedural history	
COMP rapporteur(s)	Brigitte Schwarzer-Daum / Cécile Dop
Sponsor's report submission	18 October 2024
COMP discussion	10-12 June 2025
COMP opinion (adoption via written procedure)	20 June 2025

2. Grounds for the COMP opinion

2.1. Orphan medicinal product designation

The COMP opinion that was the basis for the initial orphan medicinal product in designation in 2019 was based on the following grounds:

"Having examined the application, the COMP considered that the sponsor has established the following:

- the intention to treat the condition with the medicinal product containing nirogacestat was considered justified based on preliminary clinical data where there was an improvement in Overall Response Rate (ORR) after treatment in patients with recurrent, refractory, progressive desmoid tumours;
- the condition is chronically debilitating with a high recurrence and metastasis rate, and life-threatening with an overall 5-year survival rate of approximately 60%;
- the condition was estimated to be affecting approximately 4.6 in 10,000 persons in the European Union, at the time the application was made.

Thus, the requirements under Article 3(1)(a) of Regulation (EC) No 141/2000 on orphan medicinal products are fulfilled.

In addition, although satisfactory methods of treatment of the condition have been authorised in the European Union, the sponsor has provided sufficient justification for the assumption that the medicinal product containing nirogacestat will be of significant benefit to those affected by the condition. The sponsor has provided clinical data that demonstrate improvement in the overall response rate in patients with recurrent, refractory, progressive desmoid tumours after therapy with nirogacestat. The Committee considered that this constitutes a clinically relevant advantage.

Thus, the requirement under Article 3(1)(b) of Regulation (EC) No 141/2000 on orphan medicinal products is fulfilled.

The COMP concludes that the requirements laid down in Article (3)(1) (a) and (b) of Regulation (EC) No 141/2000 on orphan medicinal products are fulfilled. The COMP therefore recommends the designation of this medicinal product, containing nirogacestat as an orphan medicinal product for the orphan condition: treatment of soft tissue sarcoma".

3. Review of criteria for orphan designation at the time of marketing authorisation

Article 3(1)(a) of Regulation (EC) No 141/2000

Intention to diagnose, prevent or treat a life-threatening or chronically debilitating condition affecting not more than five in 10 thousand people in the Community when the application is made

Condition

Soft tissue sarcomas (STS) are a relatively uncommon group of malignancies. They comprise around 80 entities defined by the World Health Organization (WHO) classification based on a combination of distinctive morphological, immunohistochemical and molecular features. Adult-type soft tissue and visceral sarcomas (excluding GISTs) are rare tumours, with an estimated incidence averaging 4-5/ 100 000/year in Europe. (M. Bektas et al, Desmoid Tumors: A Comprehensive Review, Adv Ther (2023) 40:3697–3722). The most common STS types are liposarcomas and leiomyosarcomas (LMSs), with an incidence <1/100 000/year each, whereas the majority of sarcoma histotypes have an incidence <2/1 000 000/year. (Soft Tissue and Visceral Sarcomas: ESMO-EURACAN-GENTURIS Clinical Practice Guidelines for diagnosis, treatment and follow-up Published in 2021 - Ann Oncol (2021) Authors: A. Gronchi, A.B. Miah, A.P. Dei Tos et al., on behalf of the ESMO Guidelines Committee)

The sponsor is specifically targeting desmoid tumours (DT) which are rare, locally aggressive, fibroblastic soft-tissue tumours that are characterized by infiltrative growth and can affect organs and adjacent structures, resulting in substantial clinical burden.

They do not generally metastasize but can arise in any part of the body: extra-abdominal (appear in the head and neck, chest, extremities), and abdominal wall or intra-abdominal (originating in tissue that connects abdominal organs). Depending on their location, DT can cause debilitating pain and deformity and even life-threatening organ damage. There are two types of DT: sporadic tumours, which form the majority of all DT (85–90%) and harbour somatic b-catenin (CTNNB1) gene mutations; the remainder of DT (10–15%) occur in patients with mutations in the adenomatous polyposis coli (APC) gene, including patients with familial adenomatous polyposis (FAP), a tumour predisposition syndrome.

The course of DT is unpredictable, as spontaneous regression, stable disease, and disease progression can all occur. Depending on the location and size of the tumour and other factors, the risk of recurrence after surgical treatment can be high. Diagnosis of DT is challenging because of its morphologic heterogeneity and variable clinical presentation.

The COMP continues to designate soft tissue sarcomas to which desmoid tumours belong.

The approved therapeutic indication "*Ogsiveo as monotherapy is indicated for the treatment of adult patients with progressing desmoid tumours who require systemic treatment"* falls within the scope of the designated orphan condition "treatment of soft tissue sarcoma"

Intention to diagnose, prevent or treat

The medical plausibility has been confirmed by the positive benefit/risk assessment of the CHMP.

Chronically debilitating and/or life-threatening nature

STS is life-threatening nature with an estimated 5-year relative survival of patients with STS of any stage in Europe of 58%, and 5-year overall survival of approximately 50% (Nagar et al., 2018). STS accounts for up to 10% of all paediatric malignancies and is an important cause of death in the group below 30 years of age (Schöffski et al., 2014). An estimated 40% to 50% of patients with STS present initially with advanced STS (metastatic or unresectable locally advanced disease) and treatment options for advanced STS are limited (Nagar et al., 2018). Once soft tissue sarcoma has spread to other parts of the body, the 5-year survival rate is about 18% (Schöffski et al., 2014).

The condition is also chronically debilitating. Patients with STS often face a wide range of physical and psychological symptoms that negatively impact their quality of life (Hollander et al., 2020). Common physical symptoms in STS patients include pain, lack of energy, difficulty sleeping, and feeling bloated (Gough et al., 2017). Additionally, fatigue and muscle wasting can significantly impair daily activities of patients with STS (Gronchi et al., 2017).

Depending on their location, DTs can cause debilitating pain and deformity and even life-threatening organ damage.

The COMP has previously established that the condition is chronically debilitating due the possible need for amputation of limbs and life-threatening with a high recurrence and metastasis rate with reduced life expectancy.

Number of people affected or at risk

The sponsor has provided a prevalence estimate based on:

A review paper written by Stiller et al in 2013. In this published study, the prevalence was estimated at 4.69 per 10,000 in 2003. A few references reporting incidence were retrieved and are shown in Table 1 below. While the publications were recent, the data were not more recent than the ones used to estimate the prevalence of STS at the time of the ODD application in 2019.

Table 1. Overview of Published European incidence data for Soft Tissue Sarcoma (New Since Time of Initial ODD Application)

Author and Year of publication	Geographical region	Data source and reported period	Number of STS cases in the underlying population	Reported underlying population size	STS incidence in the underlying population (per 10,000)	Prevalence calculation [a] (per 10,000)
(Saltus et al., 2018)	Germany	Regional German cancer registries (9 registries) - between 2003 and 2012	2,635	43,530,373	0.605	4.11
(Fabiano et al., 2020)	Italy	Italian cancer registries (15 registries) - between 2009 and 2012	859	32,734,557	0.26	1.78
(Amadeo et al., 2020)	France	French network of cancer registries (FRANCIM, 19 registries) – between 2010 and 2013	1,784	Not reported	0.27 [b]	1.85

[(a)] The disease duration used for the prevalence calculation is 6.8 years based on the calculation detailed in the Sensitivity Analysis Report - Annex 3.
[b] Based on the French population in 2013 (65,600,530) as reported on Statistics | Eurostat (europa.eu).

Registry data: The Association of the Nordic Cancer Registries (NORDCAN) provided the prevalence per 100,000 inhabitants for males and females. In 2021, the male prevalence is 45 and the female prevalence 37.7 per 100,000 corresponding to 4.5 and 3.77 per 10,000 inhabitants respectively and to a mean of 4.1 per 10,000.

The International Agency for Research on Cancers (IARC) provides the most complete data collection, submitted to a rigorous methodology, for almost every European country in 2003 to 2007 (Forman et al., 2014), 2008 to 2012 (Bray et al., 2017) and 2013 to 2017 (Bray et al., 2023), reported in the Cancer Incidence in Five Continents Volumes X, XI and XII respectively. The Applicant presented the data from 2008 to 2012 in the initial ODD application and is hereby only presenting the most recent data, from the period 2013 to 2017, in Table 2.

Table 2. STS Incidence and Prevalence Data Calculated from Number of STS Cases Reported in the 27 European Member States between 2013 and 2017 from the CI5-XII report

Countries	**Reported Total Number of STS Cases Between 2013 and 2017[a]**	**Mean STS Cases per Year Between 2013 and 2017**	**Reported Size of Underlying Population Between 2013 and 2017[a]**	**Mean Incidence per Year (per 10,000) Between 2013 and 2017[b]**	**Mean Prevalence per Year (per 10,000) Between 2013 and 2017[c]**
Austria	1,564	313	8,637,112	0.36	2.47
Belgium	1,786	357	11,209,822	0.32	2.18
Bulgaria	*Data not reported*				
Croatia	719	144	4,215,907	0.34	2.33
Cyprus	95	19	854,473	0.22	1.52
Czech Republic	1,723	345	10,546,651	0.33	2.23
Denmark	1,062	212	5,669,120	0.37	2.56
Estonia	215	43	1,316,065	0.33	2.23
Finland	992	198	5,476,706	0.36	2.47
France*	2,435	495	13,672,722	0.36	2.47
Germany*	12,059	2600	59,683,127	0.44	2.98
Greece	*Data not reported*				
Hungary	*Data not reported*				
Ireland	740	148	4,695,997	0.32	2.15
Italy*	7,298	1630	44,965,567	0.36	2.48
Latvia	290	58	1,977,149	0.29	2.00
Lithuania	407	81	2,898,320	0.28	1.92
Luxembourg	*Data not reported*				
Malta	70	14	445,839	0.31	2.14
Netherlands	2,894	579	16,914,043	0.34	2.34
Poland*	144	29	1,257,845	0.23	1.56
Portugal*	23	6	246,210	0.23	1.60
Romania	*Data not reported*				
Slovakia	*Data not reported*				
Slovenia	298	60	2,062,843	0.29	1.97

Countries	Reported Total Number of STS Cases Between 2013 and 2017[a]	Mean STS Cases per Year Between 2013 and 2017	Reported Size of Underlying Population Between 2013 and 2017[a]	Mean Incidence per Year (per 10,000) Between 2013 and 2017[b]	Mean Prevalence per Year (per 10,000) Between 2013 and 2017[c]
Spain*	1,401	325	10774117	0.30	2.06
Sweden	1,624	325	9,815,302	0.33	2.26
European Union	37,839	7,981	217,334,937	0.37	2.51

*Countries with incomplete data
(a) Data extracted from the Cancer Incidence in Five Continents (CI5) report volume XII
(b) Mean Incidence per Year= (Estimated STS cases per year/ Reported size of underlying population) * 10,000
(c) Mean Prevalence per Year= Mean Incidence per 10,000 per Year x Disease Duration. Median disease duration = 6.8 years
Source: adapted from CI5 Report volume XII ((Bray et al., 2023), Annex 3).

The COMP accepted the upper estimate of 4.5 in 10,000 for STS as proposed by the sponsor. Although it is clear that patients with DT have a much lower prevalence as they only consist of 3% of STS.

Article 3(1)(b) of Regulation (EC) No 141/2000

Existence of no satisfactory methods of diagnosis prevention or treatment of the condition in question, or, if such methods exist, the medicinal product will be of significant benefit to those affected by the condition.

Existing methods

Table 3 below covers the products currently authorised in Europe for the treatment of soft tissue sarcoma.

Table 3. Authorised Medicinal Products for STS in the EU

Invented Name Active substance /INN	Indication	Route of Administration
Doxorubicin	Advanced Soft tissue sarcoma, Acute leukaemias, Hodgkin's lymphoma, Non-Hodgkin's lymphoma, Solid tumours	Intravenous injection
Eribulin (Halaven)	Liposarcoma, Metastatic Breast Cancer	Intravenous injection
Dacarbazine	melanoma skin cancer, soft tissue sarcoma, Hodgkin lymphoma	Intravenous infusion or injection
Docetaxel	Breast cancer, Non-small cell lung cancer, Prostate cancer, Gastric adenocarcinoma, Squamous cell carcinoma of the head and neck	Intravenous infusion
Trabectedin	Advanced Soft-tissue sarcoma, Ovarian cancer	Intravenous infusion

Invented Name Active substance /INN	Indication	Route of Administration
Pazopanib	Renal cell carcinoma, Soft-tissue sarcoma	Oral
Ifosfamide	Malignant disease	Intravenous infusion
Vincristine	Leukaemias, Lymphomas, Solid tumours	Intravenous infusion or injection
Paclitaxel	Ovarian cancer, Non-small cell lung cancer, AIDS-related Kaposi's sarcoma, Breast cancer, Pancreatic adenocarcinoma	Intravenous infusion

Ogsiveo as monotherapy is indicated for the treatment of adult patients with progressing desmoid tumours who require systemic treatment.

There are ESMO guidelines for this condition. (*Soft Tissue and Visceral Sarcomas: ESMO-EURACAN-GENTURIS Clinical Practice Guidelines for diagnosis, treatment and follow-up Published in 2021 - Ann Oncol (2021) Authors: A. Gronchi, A.B. Miah, A.P. Dei Tos et al., on behalf of the ESMO Guidelines Committee*)

The treatment approach for desmoid tumours varies given the unpredictable natural history of the disease (with the possibility of long-lasting stable disease and even occasional spontaneous regressions, along with a lack of metastatic potential) and functional problems implied by some tumour anatomical locations, an initial active surveillance policy can be proposed [III, A]. This should follow a careful monitoring of potentially life-threatening extra-abdominal locations (e.g. head and neck region) and intra-abdominal desmoids (mesenteric fibromatosis). Under such a policy, treatment is reserved for progressive disease. The preferred imaging modality is MRI, taking into consideration that the tumour imaging appearances may not be meaningful with regard to the disease evolution or patient symptoms.

For progressive disease, the optimal strategy needs to be individualised on a multidisciplinary basis and may consist of further watchful waiting, systemic therapies or local therapies such as percutaneous cryoablation (extra-abdominal cases) [IV, C], ILP (if the lesion is confined to an extremity) [IV, C] and surgery in favourable locations (i.e. abdominal wall) [IV, C]. Definitive RT should be considered after multiple failed lines of treatment or for tumours in critical anatomical locations where surgery would involve prohibitive risk or functional impairment, especially in elderly patients [III, C]. When a systemic therapy is chosen, available options include low-dose ChT (such as methotrexate-vinblastine or methotrexate-vinorelbine; oral vinorelbine; taxanes); sorafenib [II, B]; pazopanib; imatinib; and full-dose ChT (using regimens active in sarcomas, including liposomal doxorubicin). In addition, HT (tamoxifen, toremifene and GnRH analogues; aromatase inhibitors), nonsteroidal anti-inflammatory drugs and interferon have also long been used, but no prospective studies are available to understand their real activity in this disease.

Although none of the products in the table above are authorised specifically for desmoid tumours, products approved for the broad indication "soft tissue sarcoma" can be considered satisfactory.

Significant benefit

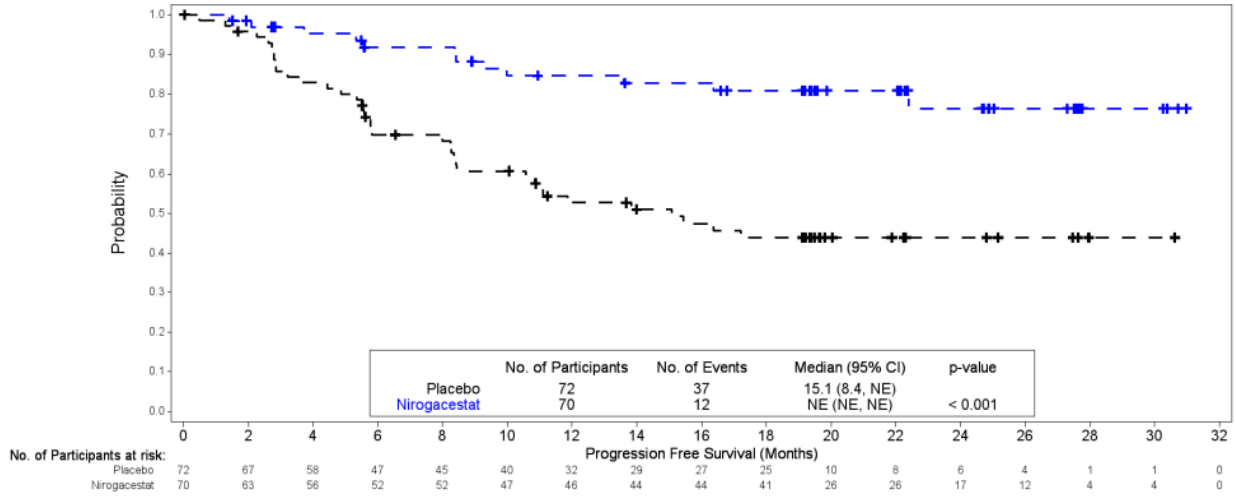
The sponsor believes that their product will be of significant benefit as it offers a clinically relevant advantage after prior lines of systemic treatment.

The sponsor has provided data from their Study NIR-DT-301 is a randomised, DB, placebo-controlled, Phase 3 study to evaluate the efficacy, safety, and tolerability of nirogacestat in adult participants with progressing DT. This study consists of 2 phases: a DB phase and an optional OLE phase. The primary analysis for the DB phase was performed utilizing a 07 April 2022 data cut with final database lock occurring on 30 June 2022; the OLE phase is currently ongoing. Nirogacestat was administered orally at a dose of 150 mg BID continuously, in 28-day cycles.

As of primary analysis, 07 April 2022, a total of 142 patients were randomised to nirogacestat 150 mg BID (n=70) or placebo (n=72) and the study met its primary endpoint.

Median PFS was not estimable in patients who had received nirogacestat 150 mg BID as only a small proportion of participants experienced disease progression during follow-up, compared to 15.1 months in the placebo arm (p<0.001). A statistically and clinically significant improvement in PFS was observed for nirogacestat over placebo, with a 71% reduction in the risk of disease progression or death (HR = 0.29; 95% CI: 0.15, 0.55; p < 0.001). The probability of being event-free at 12 and 24 months is consistently higher for nirogacestat as compared to placebo, supporting a sustained clinical benefit over time (Figure 1).

Figure 1. Kaplan-Meier Plot of PFS from Randomization – Double-Blind Phase (ITT Population)



Note: Progression free survival was calculated as: (date of death or [radiographic/qualified clinical] progression or censoring date – randomization date + 1)/30.4375. Censoring was defined in Section 9.7.3.1.1.
Note: Qualified clinical progression events were clinical progression events assessed by the investigator that were adjudicated by an independent Endpoint Adjudication Committee.
Note: Median and 95% confidence intervals were estimated from the Kaplan-Meier method.

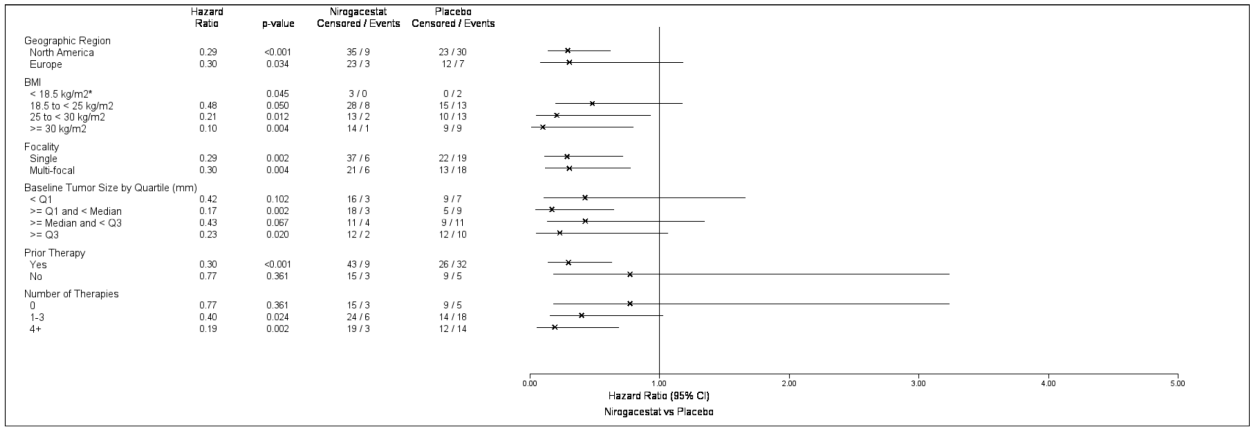
The sponsor conducted a subgroup analysis as summarised in the table 4 below.

Table 4. Subgroups for Efficacy Analysis

Stratification	
Stratification factor as reported in randomization	
Demographics	
Sex (Male vs Female)	Age (by quartile)
Race (White vs Non-White)	Ethnicity
Geographic region (North America vs the rest of world)	BMI (18.5 kg/m^2,18.5 - < 25 kg/m^2, 25 - < 30 kg/m^2, ≥ 30 kg/m^2)
Disease Characteristics	
Multi-focal disease vs single tumor	Baseline target lesion size by quartile
Baseline target lesion locations[1]	
Prior Treatment	
Any prior therapy (Yes vs No)	Number of prior lines of therapies (0, 1-3, 4+)
Prior systemic therapy (Yes vs No)	Prior surgical treatment (Yes vs No)
Prior radiation treatment (Yes vs No)	Previous exposure with sorafenib (Yes vs No)
Prior chemotherapy exposure (Yes vs No)	Prior tyrosine kinase inhibitor exposure (Yes vs No)
	Desmoid tumor treatment status[2]
Dose Modification	
Dosed per protocol vs reduction (Yes vs No)	Relative Dose Intensity (≤ 80% vs > 80%)
Genetic Mutation	
History of familial adenomatous polyposis (FAP)	Presence of any CTNNB1 mutation, somatic CTNNB1 mutation, or germline CTNNB1 mutation
Presence of any APC mutation, somatic APC mutation, or germline APC mutation	
Adverse Event	
Highest Reported FSH in women of childbearing potential (WOCBP) by range indicator (Low/Normal, High)	WOCBP with events of ovarian dysfunction (as defined by a narrow list of terms per Section 7.5.1) that have resolved versus those that have not resolved
Participants with AEs of Rash or Alopecia (as defined by all narrow terms in Section 7.5.2).	Participants with AEs of Diarrhea within the first 3 cycles

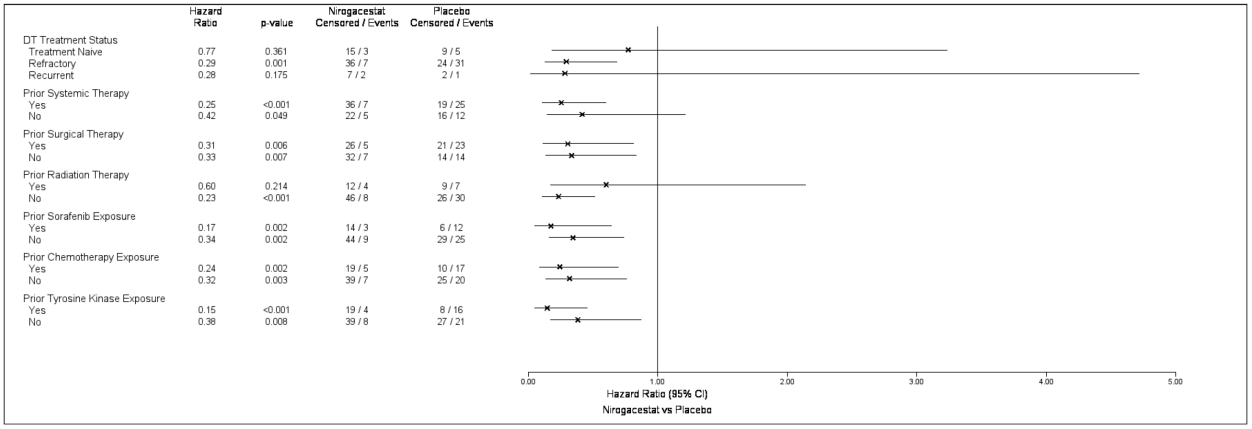
[1] Baseline target lesion location is based on actual target tumor location from the Electronic Database. Baseline target lesion locations: Intra-Abdominal (including mesentery and pelvis) and Extra-Abdominal (including head/neck, para-spinal, extremities, abdominal/chest wall, and other locations). If a participant has multiple target tumors that are located in both the intra and extra-abdominal location, the tumor will be classified as intra-abdominal.
[2] Desmoid tumor treatment status: 1) Treatment naïve, measurably progressing DT/AF, 2) Recurrent, measurably progressing DT/AF following at least one line of therapy, and 3) Refractory, measurably progressing DT/AF following at least one line of therapy

Figure 2. Number of prior therapies



In Figure 3 it can be seen that when patients have had one or more lines of therapies the PFS continues to be favourable for the sponsor's product.

Figure 3. Type of prior therapy



Participants who failed prior treatment with sorafenib, still reported a significant improvement with nirogacestat as compared to placebo (HR of 0.17, $p<0.002$)

Subgroup analyses showed similar PFS results across all prespecified subgroups including demographics (gender, race, region), disease characteristics (single tumour/multi-focal), prior treatment, gene mutations (history of FAP, presence of any AFP mutation) and adverse events.

The treatment option landscape for STS and DT has not evolved since the orphan designation in 2019. For patients that have relapsed or recurrent DT, or for patients with DT that are not amenable to surgery or are symptomatic, various medical interventions have been studied (e.g., hormonal therapy, NSAIDs, chemotherapy, and targeted therapy); although very few in controlled clinical studies. While active treatment with surgery, radiotherapy, and/or systemic therapy with hormonal therapy, NSAIDS, chemotherapy, or tyrosine kinase inhibitors may be indicated in patients with persistent DT progression, there is no accepted standard-of-care for DT patients. There are significant limitations associated with these alternative therapeutic approaches for DT including a high rate (up to 70%) of disease recurrence associated with surgery (Bonvalot et al., 2012), an increased risk of radiation-induced neoplasms associated with radiotherapy (B. Kasper et al., 2017b; B. Kasper et al., 2011; Ray et al., 2006), and a lack of efficacy and / or tolerability associated with systemic therapies (Alman et al., 2020). In addition, even when these alternative therapies are used, patients with DT often progress. Nirogacestat has shown clinical benefit in patients who have failed multiple prior treatments for their DT (Kummar et al., 2017).

The sponsor provided data showing that nirogacestat is superior to placebo, based on PFS and ORR, in patients who received previous chemotherapy.

The COMP considers this a clinically relevant advantage.

4. COMP position adopted on 20 June 2025

The COMP concluded that:

- the proposed therapeutic indication falls entirely within the scope of the orphan condition of the designated Orphan Medicinal Product.
- the prevalence of soft tissue sarcoma (hereinafter referred to as "the condition") was estimated to remain below 5 in 10,000 and was concluded to be 4.5 in 10,000 persons in the European Union, at the time of the review of the designation criteria;
- soft tissue sarcoma is chronically debilitating with a high recurrence and metastasis rate, and life-threatening with an overall 5-year survival rate of approximately 60%; desmoid tumours are chronically debilitating due to pain, loss of function in the affected area and cramps and nausea, when desmoid tumours occur in the abdomen.
- although satisfactory methods for the treatment of the condition have been authorised in the European Union, the claim that Ogsiveo is of significant benefit to those affected by desmoid tumours, a subset of soft tissue sarcomas, as defined in the granted therapeutic indication is established. Ogisveo has shown statistically and clinically relevant improvements in progression free survival compared with placebo, in patients who received previous treatments approved for the broad indication soft tissue sarcoma.

The COMP, having considered the information submitted by the sponsor and on the basis of Article 5(12)(b) of Regulation (EC) No 141/2000, is of the opinion that:

- the criteria for designation as set out in the first paragraph of Article 3(1)(a) are satisfied;
- the criteria for designation as set out in Article 3(1)(b) are satisfied.

The Committee for Orphan Medicinal Products has recommended that Ogsiveo, nirogacestat for treatment of soft tissue sarcoma (EU/3/19/2214) is not removed from the Community Register of Orphan Medicinal Products.