



Info.suppl.



## The role of patients' associations

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Received 17 March 2019

Accepted 20 June 2019

Available online: 4 December 2019

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**Keywords**Desmoid tumor  
SOS DESMOÏDE  
Patient group  
Netsarc  
Perseus  
Rare disease**Summary**

The role of patient groups has grown steadily stronger since the first General Meeting of cancer patients in 1999 and the emergence of the rare diseases issue in the mid-eighties. This article demonstrates the role of a patient group (SOS DESMOÏDE) gathering people suffering from a rare disease (the desmoid tumor) in the stimulation of scientific and medical research as well as the improvement of patient care, information and support provided to patients. It substantiates these elements with numerous socio-medico-psychological surveys, scientific publications and medical research implemented by medical teams and supported, even sometimes initiated, by SOS DESMOÏDE. This research effort illustrates the significant impact of a partnership between patients and physicians-researchers on medical and scientific breakthroughs in a specific pathological field.

**Mots clés**Tumeur desmoïde  
SOS DESMOÏDE  
Association de patients  
Netsarc  
Perseus  
Maladie rare**Résumé****Le rôle des associations de patients**

*Le rôle des associations de patients n'a cessé de se renforcer depuis les premiers états généraux des malades du cancer en 1999 et l'émergence de la problématique des maladies rares au milieu des années quatre-vingts. Cet article illustre le rôle d'une association de patients (SOS DESMOÏDE) atteints d'une maladie rare (la tumeur desmoïde) en matière de recherche scientifique et médicale mais aussi en termes d'amélioration des prises en charge, de l'information et du soutien aux patients. Il est étayé par de nombreuses enquêtes socio-médico-psychologiques, publications scientifiques et travaux médicaux mis en œuvre par des équipes médicales et soutenues, parfois même initiés, par l'association SOS DESMOÏDE. Ce travail de recherche illustre l'impact considérable que peut avoir un partenariat entre des patients et des médecins-chercheurs sur les avancées médicales et scientifiques observées dans un domaine pathologique donné.*

**A** catalyst is either something that makes a chemical reaction happen more quickly without itself being changed, or an event or person that causes great change. This is the role of patient groups like SOS DESMOIDE.

SOS DESMOIDE was born in 1998 thanks to a young patient who wanted to help her oncologist to find a treatment for her disease. Faced to her doctor's doubts regarding her decision to create a patients' association, when she was hoping for a doctors and researchers network, she declared: « As Perseus against Medusa, we will use the tumor's own weapons to defeat it. We will branch out! Do you want to be part of the adventure and be the Secretary General? ». And this is how, with only one identified patient, in the very beginning of the internet, when the first General Meeting of cancer patients had not taken place yet, they addressed the challenge.

Today, after twenty years of works in partnership with doctors and researchers, SOS DESMOIDE improved significantly visibility on the desmoid tumor and on the patients who suffer from it. The association offered patients, and parents of patients, an adequate structure and informative documents [1,2] to break their isolation and give them better guidance. This individualized orientation is taken in charge thanks to a partnership with the "Rare Diseases Info Services" (MRIS) platform through a hotline and a forum. It also stimulated medical research to accelerate diagnosis and make it more reliable, to better understand the evolution mechanisms of the pathology and hence to improve the patients' journey. SOS DESMOIDE has always worked starting from patients' questionings and perceptions because it considers them as a true expertise that complements the one doctors possess.

This strategy, based on complementarity between patient doctor, original and innovative at the time, impacted very significantly these past twenty years findings in France. It required shaking up conventional thinking and breaking the vicious circle of rarity: rare disease, unknown, hence with limited visibility and not arousing researchers' interest, hence bringing neither research projects nor research budgets. A sustained ignorance in fact. SOS DESMOIDE managed to break this circle by convincing Orphanet [3] to publish an article in its guide on rare diseases, then by building a tissue collection with the help of the Tissue Bank for Research (BTR) [4] and of a pathologist from Bordeaux [5]: a convention for the constitution of collections of biological samples was signed in 2014 at the Pitié-Salpêtrière hospital in Paris between the BTR, the French Association against Muscular Dystrophy (AMF) and the association SOS DESMOIDE. Last but not least, by following the advice of its first Secretary General, the association integrated the referral network for soft tissue sarcoma. Ten years later, SOS DESMOIDE rallied to its cause a community of doctors and allowed patients to earn several years of diagnosis delay by giving them a network of expert centers in our disease care (NETSARC) [6,7].

SOS DESMOIDE had still the wish to direct research by relying on patients' perceptions. The association first started with the impact of surgery on the desmoid tumor evolution, a major subject that was about to change dramatically the disease care. In 1998, desmoid tumors were always treated by surgery and then radiotherapy. Patients were experiencing mutilating surgeries, causing permanent disabilities. With an interview outline approved by patients, a dozen surgeons were interviewed and reacted, sometimes very vigorously, to patients' perceptions of an aggravation of their disease following the surgery. One surgeon addressed the challenge and proposed an extensive research program to evaluate surgery impact on the evolution of desmoid tumors. A phase II study financed by a Hospital Clinical Research Program (PHRC) [8] led to an active observation strategy to replace surgery as first-line treatment ! SOS DESMOIDE achieved another goal: thanks to the support of an established researcher in molecular biology, the French National Cancer League (LNCC) accepted to integrate the desmoid tumor in the cancer program Identity Card of Tumors (CIT). This big step led us in 2008 to the first European research program in molecular biology, PERSEUS [9-15], initiated by SOS DESMOIDE, supported by Professor JM Coindre based on a first project drafted in 2001 with Dr M Longy, and funded by the LNCC in the framework of CIT. This project implemented by the network's doctors and researchers of the French Sarcoma Group (GSF) and CONTICANET (CONnective Tissue Cancer NETwork: European network of excellence on conjonctive tumors) gave birth to numerous publications [9-15].

To maintain momentum, SOS DESMOIDE committed in the Rare Diseases Alliance, Rare Diseases Europe (EURORDIS), CONTICANET, Sarcoma Patients European Network (SPAEN) [16], Intersarc and in the polyposis and gastroenterologists network. The association regularly organizes medico-scientific seminars and calls for projects, particularly relayed by the Intersarc intergroup and the GSF-GETO [17,18], that support new leads of research, which brings it to evaluate projects and contribute to the design of clinical trials like CRYODESMO [19] ou DESMINIB [20,21] by questioning their rationale from the patients' perspective. Other examples: SOS DESMOIDE offered financing of 5000€ for a proof of concept on the prognostic value of the plasmatic concentration of circulating cell free DNA in desmoid tumors' proliferation [22], of 7500€ for the project called « Desmoid tumors and functional sequelae for children » [23], of 15000€ as seed money for an ongoing project aiming at evaluating the correlation between specific markers and the existence of an intra-abdominal desmoid tumor [24], of 15000€ for work on the disease representations and the subjective experience of patients suffering a desmoid tumor [25], and allocated lately a 45000€ contribution to a thesis financing aiming at building a mouse model for desmoid tumors [26]. All the funding granted by SOS DESMOIDE comes from donations of patients and members and from events organized by the association and its

supporters like village fairs for example, flea markets, sports tournaments, and also the « worldtour » (each participant collects donations for each kilometer, with the objective to reach altogether the world tour). SOS DESMOIDE supports in an institutional and operational way some projects as well in order to help them obtaining major funding, like for example for the ALTITUDES Protocol [27]. The progress made and the collected data, particularly via surveys managed by SOS DESMOIDE [28], are shared every year with patients, members and doctors who are involved during the Annual Meeting, the cornerstone of our permanent support framework to patients. This framework includes notably the psychological support unit « DesmoPsy » established as early as 1999, a weekly shift on the « Desmoid tumor » forum of MRIS, the organisation of meeting days for patients in regions and the yearly workshops « Patients' word » hosted by psychologists. The Annual Meeting is also an opportunity to strengthen the patient-doctor partnership that made the patient group's success, whose governance is still mixed nowadays: the chair is held by two patients and the Secretary General by an oncologist and a pediatric oncologist. It is the same for active members of the associative group composed of eight patients (including two children who have always been members and joined the Group once adults), three parents of patients, two relatives of patients and four doctors. We are all convinced of the complementarity of our knowledge and expertise, expert patients even sometimes participate in caregivers' training like in the Institut Bergonié where groups of patients

and parents of patients, including SOS DESMOIDE, animated a slot called « Role of companions for teenagers or young adults in building process » in the framework of a training organized by the EFEC (UNICANCER). This patient-doctor partnership is truly the keystone of SOS DESMOIDE.

To push even further, the association now animates a research unite (called task force), with the involvement of oncologists and expert researchers in our disease, coming from Bordeaux, Lille, Lyon, Marseille, Paris, Strasbourg and Toulouse, that will write the next pages of SOS DESMOIDE's path to an eventually effective treatment and a long term less noxious health care for this rare chronic disease.

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**Disclosure of interest:** the authors declare that they have no competing interest.

### Supplementary data

Supplementary data associated with this article can be found, in the online version, at <https://doi.org/10.1016/j.bulcan.2019.06.017>.

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