RARE CANCERS: CRACKING THE CODE

SPECIAL REPORT | 7 - 13 FEB 2017
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Rare cancers: A headache for policymakers
Andriukaitis: ‘Rare cancers present a particular challenge’
Complex sarcoma cancer needs an overall strategy, report says
How Italy and France take on rare sarcoma cancers
Health matters should supersede European bureaucracy
The complexity of diagnosing and treating rare forms of cancers like sarcomas can be addressed by a multi-level approach and better coordination among member states, insist experts in the field.

The increasing occurrences of rare diseases has raised concern in Europe and policymakers are now stepping up efforts to address the situation.

Some five to 8,000 rare diseases currently affect 6-8% of the population in the EU today – between 27 and 36 million people – according to estimates.

Rare cancers, those with fewer than five cases out of 10,000, belong to this category. It may sound small, but according to European Commission estimates, this adds up to millions of people across the EU who are affected by such types of cancers.

Speaking at the kick-off meeting of the Joint Action of Rare Cancers in Luxembourg last November, EU Health and Food Safety Commissioner Vytenis Andriukaitis admitted that rare forms of cancer were particularly difficult to address and that there is no straightforward solution.

He said that 186 different types of cancer, including all childhood cancers, affected only a small number of patients each.

“Together, however, they affect over 4 million people and account for 22% of all cancers diagnosed in the EU each year,” the Commissioner noted.

WHAT ARE SARCOMAS?

Sarcomas are a diverse family of rare cancers that develop in connective tissues that can affect virtually any part of the body. But because so many different types of sarcomas exist, the diagnosis, treatment, research and policy-making surrounding them is challenging.

In the EU, it is estimated that there are 5.6 cases per 100,000 annually, representing 27,908 new cases per year.

For over 30 to 40 years there have been no significant advances in sarcoma treatments while effective treatment options are limited, especially for soft tissue cancers.
sarcomas, which make up 84% of cases (bone sarcomas make up 14%). However, experts have noted that gastrointestinal stromal tumours (known as GIST) are an important exception, and have seen treatment advancements in recent years.

As a consequence, the survival rate is 47% for rare cancers versus 65% for common cancers.

According to Denis Horgan, executive director of the European Alliance for Personalised Medicine, the burden of rare diseases, such as sarcoma, on society is getting larger and should be prioritised on the political agenda.

“Some governments have begun to develop special access programmes, such as compassionate use, which increases early access to so-called orphan drugs,” Horgan told Euractiv.com.

“This is to be welcomed but is not yet enough. The growing prevalence of rare diseases should make the area a healthcare priority for Europe, now and into the future,” he said.

THE DIAGNOSIS BURDEN

The chances of surviving sarcoma basically hinge on how early the disease is diagnosed.

Experts claim, though, that the diagnosis in this particular case is problematic, as non-specialists often can’t identify sarcomas due to their rarity, and inaccurate diagnosis leads to mismanagement in 70% of patients.

In addition, a late or inaccurate diagnosis results in high costs and poor quality of life for sarcoma patients and their families, while some treatments are not reimbursed, so patients may have to travel long distances for care and pay out-of-pocket.

Sarcoma patients often lack information about their condition, treatment, and ongoing clinical trials, and as a result, can’t make informed care decisions.

Dr Wendy Yared, Director of the Association of European Cancer Leagues (ECL), told Euractiv that improving the early diagnosis and timely referral of sarcoma cases is a major issue.

“Because sarcoma is so rare; because there is such a large number of types; and because symptoms are nebulous such as lumps and pain, proper diagnoses require quality imaging and biopsy services provided by specialised radiologists and pathologists,” she emphasised, adding that reference centres specialising in sarcomas could play a crucial role.

Dr Yared also called for closer collaboration between all relevant stakeholders – cancer leagues, lawmakers, patient organisations, academia, industry, and health professionals – in order to ensure that “true gaps and realistic solutions are identified”.

BETTER TRAINING

Boosting professional training is another major challenge, as sarcomas can occur anywhere in the body, meaning any type of physician may discover them.

But the problem is that most will not have seen a sarcoma and may not know what specialist to refer their patient to, leading to a delayed diagnosis, or a misdiagnosis.

In addition, many oncologists haven’t been trained on how to diagnose or treat sarcomas.

Professor Stefan Bielack, a board member of the European Society for Paediatric Oncology (SIOPE), told Euractiv that suitable life-long training and education for primary care practitioners is crucial, as most of them are unfamiliar with the symptoms.

“Primary care needs to be aware of the possibility that a mass may represent a sarcoma,” the professor said. “Otherwise the correct diagnosis will be delayed, and this delay may negatively impact on a patient’s probability of survival.”

THE RESEARCH CHALLENGE AND ORPHAN DRUGS

Professor Bielack, also, stressed that almost no drug would be exclusively developed for rare cancers, such as any of the individual sarcoma subtypes.

“Therefore, schemes making drugs which were developed for the more common cancers available to sarcoma patients must be implemented, and these must reliably perform,” the professor emphasised.

The Association of European Cancer Leagues (ECL) recently established a European Task Force for Equal Access to Cancer Medicines during the Dutch EU Presidency.

The task force insists that patients should be able to access all effective and innovative cancer treatments.

“Specific actions are still being defined by Netherlands, France, member leagues of the Task Force, but suggested solutions may well indeed require a call on regulatory pathways to be more flexible,” Dr Yared told EurActiv.

In an effort to help lawmakers tackle the complex challenges related to Sarcoma care, in recent months, a multi-stakeholder group of experts has been developing the Sarcoma Policy Checklist, which is due to be presented during an event on Wednesday (8 February) in the European Parliament.
The European Commission’s priority when tackling rare cancers such as sarcomas is to ensure European Reference Networks (ERNS) are put in place so that all patients have access to the best expertise available in the EU, Vytenis Andriukaitis told Euractiv.com in an interview.

Vytenis Andriukaitis is EU Commissioner for Health and Food Safety.

He spoke with Euractiv’s Sarantis Michalopoulos.

You recently warned that “the clock was ticking on an oncological time bomb”. What has the Commission already done to address the situation and what is next?

The EU has been fighting cancer for 32 years, taking a multifaceted approach which covers prevention, screening, research and more. Actions taken at EU-level have helped to extend and save lives, but more needs to be done.

As at least one in three cancers is preventable, our first line of attack is to address the risk factors. This is achieved not only through EU legislation on, for example, pesticides, air quality, tobacco products and exposure to carcinogens at work, but also through platforms and projects to address alcohol consumption, unhealthy diets, and obesity.

The Commission has also produced guidelines for quality screening of breast, cervical and colon cancer, provided nearly €1 billion for collaborative research and supported member state efforts to improve diagnosis and treatment, among many other actions.

For a full overview, I invite you...
Continued from Page 6

to see our latest info on EU action on cancer.

However, rare forms of cancer such as sarcomas, present a particular challenge in terms of ensuring all patients have access to the best expertise available in the EU. Therefore, going forward, my priority will be to ensure European Reference Networks (ERNs) for rare and complex diseases are a success. They will be in operation in March 2017.

It is particularly important that we leave no one behind in our efforts to help cancer patients get the best possible screening and care. People with lower income or lower education do not have the means or the knowledge to lead a healthy life or go to the doctor for screening, and end up in a hospital when their cancer is already at an advanced stage.

To address cancer, we therefore, need national health systems that reach out to the poorest and address social inequalities and social determinants in cancer prevention, access to screening and care. We need pro-active and wide-ranging onco-policies with a greater focus on prevention, prophylaxis and long-term patient monitoring policies; as well as greater use of prevention in primary care. I am ready to support member states in such efforts.

How could EU policymakers and the member states address rare cancer cases considering the lack of pharmaceutical sector interest in developing new orphan drugs for such a small number of patients?

Patients suffering from rare diseases and conditions should be entitled to the same quality of treatment as patients affected by other diseases, which is why in the year 2000, the EU passed specific legislation to incentivise the development of orphan drugs. This instrument has helped to stimulate interest and has led to developments including for some rare cancers.

We have to be realistic, however. There are several thousand rare diseases, and even the best policy instrument cannot ensure simultaneous progress in all therapeutic areas.

But we also have every reason to be optimistic. Many new cancer drugs may work not only for one type of cancer but for several. Even if a drug is initially developed just for one subtype, the product could potentially, over time, be developed for use in other rare cancers, such as sarcoma.

What could be the practical added value of the European Reference Networks for patients suffering from rare cancers?

There are some rare, genetic forms of cancer which are extremely difficult to diagnose and treat, and sadly, many of them affect children. Such rare forms of cancer represent a particular challenge as specialist knowledge is often frustratingly scarce, and patients and parents are sometimes left to scour the internet to find doctors and centres with the required expertise.

No country alone has the knowledge and capacity to treat all forms of rare cancer, but by cooperating and exchanging life-saving knowledge at European level through European Reference Networks (ERNs), patients across the EU will have access to the best expertise available.

For example, the recently approved ERN on rare and low prevalence and complex Cancer in Adults (ERN-EUROCANC) will give healthcare providers access to a much larger pool of expertise and knowledge, increasing the chances of patients suffering from such conditions and diseases to receive the best advice to treat and diagnose their condition.

Considering the complexity of different rare cancers types, accessibility and affordability of medicines are tough challenges for patients. A particular drug can be found in one member state while in another one it does not exist at all. Does this go against the spirit of the EU internal market?

Products that are authorised under the orphan medicines regulation receive a marketing authorisation [by the Commission], which is automatically valid in all member states. Hence, the marketing authorisation is not the key issue. Accessibility and availability are rather linked to other issues, such as costs, pricing, and reimbursement and the speed of market introduction.

But don’t get me wrong. Accessibility and affordability are ones of the main concerns of the EU, which is also demonstrated by the recent Council conclusions on the topic. We are in a continuous dialogue with member states, the European Medicines Agency, national competent authorities, and industry in order to see how we can support accessibility and affordability. There is no magic solution. A constant effort is required to improve the situation and to ensure that patients have early access to promising new therapies.

To improve access for patients to truly innovative therapies, I am convinced that the pan-EU cooperation on health technology Assessment (HTA) we are working on will bring real added value to all countries, through the pooling of resources, exchange of expertise, and the avoidance of duplication in the assessments of the same product or intervention in different member states. Multiple assessments represent delays for patient access to innovative treatments.

Continued on Page 8
What is the role of patient organisations in tackling rare diseases? Do you believe they have been sufficiently enhanced during the last few years?

Empowering patients’ organisations is a cornerstone of the EU rare diseases policy and one of the seven priorities of the 2009 Council recommendation on an action in the field of rare diseases. Patients’ organisations are very important, active and constructive members of all relevant groups and committees dealing with issues related to rare diseases policy. A few examples, amongst others, are the Commission expert group on rare diseases and several EMA committees (Committee for Orphan Drugs, Paediatric Drugs Committee). The Commission also supports rare diseases patients’ organisations through operating grants from the EU Health Programme.

Sarcoma is rare cancer and one of its main challenges is the lack of training in identifying the symptoms. This results in a poor or even wrong diagnosis. What could be a European policy response to the training issue?

Adequate medical training is essential for exploiting knowledge and expertise on rare cancers and improving patient outcomes. It is well known that rare cancers such as sarcoma present special challenges exactly because finding clinical expertise in the community is difficult.

ERNs have the specific objective to exchange, gather and disseminate knowledge, evidence, and expertise within and outside the Networks, and can, therefore, bring about big improvements in this area.

It is clear that medical education should be dealt with differently for common cancers and rare ones. With the more frequent cancers, transfer of knowledge is automatically reinforced in the everyday practice of healthcare professionals. This is not the case for rare cancers, however, except in reference centres.

The Joint Action on Rare Cancers (JARC) launched by the European Commission in 2016 also aims to address the issue of training. It includes a specific Work Package on Medical Education which aims at mapping existing educational resources on rare cancers and at identifying optimal ways to promote medical education in the context of ERNs.

Are you concerned about the growing migration of doctors from poorer to wealthier EU member states? Is this another obstacle to a common approach toward rare diseases?

Free movement in the European labour market offers valuable job and training opportunities for health professionals to develop their expertise, also in the area of rare diseases. While there are no easy solutions to the high outflows of doctors from some member states, the EU can support the transfer of good practices and knowledge across borders to improve access to healthcare. ERNs are a good tool to transfer knowledge between member states. This can be done through education and training partnerships and exchanges of medical specialists between ERNs, for example.

Do e-health solutions have a place in tackling rare diseases considering the heterogeneity of most cases?

The current trends in eHealth and ICT solutions in Europe are to move towards interoperable solutions that enable patient data sharing, state-of-the-art data protection and common clinical platforms and tools, involving the most advanced ICT tools and services.

Most of the tools developed under the general concept of eHealth would be of key relevance for the structure of ERNs, and would assist them in their work to better diagnose and treat patients with rare cancers and other rare diseases.

In fact, the implementation of ERNs is one of the most important and innovative pan-EU initiatives for cooperation in eHealth, and ensuring that this cooperation reaches its full potential is one of my key priorities for 2017. These ERNs will be supported by telemedicine tools, and we will ensure that ERNs receive support via a range of EU funding mechanisms such as the EU Health Programme, the Connecting Europe Facility and the EU research programme Horizon 2020.
The diverse nature of rare cancers like sarcomas poses severe challenges to the medical community and policy-makers who need to seek a holistic approach, according to a report.

Sarcomas are a heterogeneous family of rare cancers. Due to poor diagnosis and low awareness among health practitioners, they pose a particular challenge to both patients and policymakers.

In the EU, it is estimated that there are 5.6 cases per 100,000 annually, representing 27,908 new cases per year [See background].

**NEED FOR EU-WIDE COOPERATION**

Speaking at the “European policy response to rare cancers: the case of sarcoma” event yesterday (8 February), EU Commissioner for Health and Food Safety Vytenis Andriukaitis presented the executive’s work on rare diseases and cancers and called for targeted policies built on solid European cooperation.

“People with rare cancers such as sarcoma can face many years of suffering and uncertainty, misdiagnosis, with no access to appropriate treatment and care.

Continued on Page 10
Helping people with rare cancers get the best possible diagnosis and care across the EU is our common goal,” the EU health chief said, adding that the “Social Pillar” of the EU must address those issues.

**SARCOMA POLICY CHECKLIST**

In an effort to help policy makers tackle the complex challenges related to Sarcoma care, a multi-stakeholder group of experts developed the Sarcoma Policy Checklist and made several recommendations.

One suggestion is to establish national centres of reference on sarcoma working with a European Reference Network (ERN) in order to combine resources. Centralising care in high-volume centres has been shown to improve the overall quality of care for sarcomas, the report found.

According to the Commission, ERNs for rare diseases serve as research and knowledge centres, updating the latest scientific findings, treating patients from other member states and ensuring the availability of subsequent treatment facilities where necessary.

**BETTER TRAINING**

Another recommendation is to boost professional training, as sarcomas can occur anywhere in the body, meaning any type of physician may discover them. But the problem is that most will not have seen a sarcoma and may not know what specialist to refer their patient to, leading to a delayed or misdiagnosis.

In addition, many oncologists haven’t been trained on how to diagnose or treat sarcomas.

The Sarcoma Patient Experience Survey in England found 27% of patients who saw a general practitioner (and 25% who went to a hospital) for sarcoma symptoms “were started on treatment for another condition or were told symptoms were not serious”.

Estelle Lecointe, a representative of the Sarcoma Patients EuroNet (SPAEN) and Info Sarcomes, said that the first challenge a sarcoma patient is faced with was getting the right diagnosis.

“Sometimes, it can take weeks or even months, especially in some countries, before getting the right diagnosis due to the fact that these tumours are so rare that non-specialists have never seen a sarcoma patient case,” she noted, adding that these tumours can look benign and are confused with non-malignant diseases.

She continued, saying that there are countries, which have no expert networks, and as a consequence patients cannot find the proper doctor. “It’s also difficult for these doctors to be in touch with physicians who have the competence to guide them regarding the treatment or at least to refer them to a specialist,” she added.

ERNs, according to Estelle, are a move in the right direction. “They can significantly contribute by promoting education programmes to train non-specialist doctors especially from countries, where there are absolutely no experts networks.”

**RESEARCH CHALLENGE AND ORPHAN DRUGS**

The report also found a lack of funding for basic research on sarcomas and called for more incentives to support public-private partnerships focused on rare cancers. In addition, patients receiving treatment in reference centres were found to have a limited access to clinical trials.

The UK’s national sarcoma survey found 67% of patients were not asked whether they wanted to take part in a trial, and if they were, only 22% participated. Low enrolment in
Continued from Page 10

sarcoma trials is a problem due to the small numbers of patients with each type of sarcoma, meaning data collection is critical for any progress to be made, the report noted.

It added that a national clinical trial portal listing all ongoing sarcoma clinical trials should be made available to the public.

The authors of the report also underlined the many hurdles standing in the way of developing drugs for rare cancers, due to their rarity.

Estelle pointed out that access to sarcoma related medicines in some countries is a remarkable obstacle and healthcare systems cannot afford them. “They are really expensive, more than €2,000 per month, the less expensive [...] and they cannot be reimbursed because the patients are so few,” she noted.

Describing the complexity of sarcomas, she said that even with the same diagnosis one could have molecular sub-types, which will not have the same reaction to the drugs.

We should change the policy and develop the expert network centers, it should be the right of every patient to receive the right expertise and for the moment only few countries have this and that creates inequalities among patients.

The report underlined that patient involvement in Health Technology Assessment (HTA) and other access pathways is key to ensuring new treatments are developed based on what is most important to patients.

Since it is difficult to enroll patients in clinical trials, the report says physicians should have access to patient-friendly online portals that list all ongoing sarcoma clinical trials.

The HTA is a multidisciplinary process to assess the added value and effectiveness of a given health technology – for example medicine, medical devices, diagnostic tools or surgical procedures, over and above existing ones.

THE PHARMA INDUSTRY CHALLENGES

Dr Nora Drove Ubreva from Lilly pharmaceutical company stressed that particularly for sarcoma disease the pharma industry was “lacking expertise”.

“We are lacking registries, which would allow us to learn more about the disease and initiatives”, she explained, adding that the complexity of sarcomas makes planning and conducting clinical research “much harder”. To fully encompass a large number of sarcoma types, clinical trials should be conducted on an international scale, she said.

There is also a “disconnect” between what types of data that regulators and HTA bodies are asking for, Drove said. Regulators tend to be more flexible when it comes to conditional approval and allowing quicker access, while HTA bodies have been slower to adapt.

“Sometimes they are asking for data that may take years to generate, which is extremely complex,” she said. “This not only makes for difficult clinical research, it also delays access for patients to drugs that they really need.”
How **Italy** and **France** take on rare **sarcoma** cancers

**EU** member states are exploring ways to effectively tackle the rising occurrence and complex nature of rare cancers known as sarcomas.

Sarcomas are a particular group of rare cancer which, due to a low number of patients, has so far attracted little attention.

But this might change. A multi-stakeholder group of experts recently developed the Sarcoma Policy Checklist, an inventory of recommendations aimed at better managing sarcoma cases in Europe.

Six EU countries (Sweden, Germany, Spain, Italy, France and the UK) were profiled in the Sarcoma Policy Checklist, suggesting concrete steps for national authorities to take in order to improve sarcoma care.

Italy and France both provide interesting examples of national improvement efforts aimed at strengthening sarcoma care, in alignment with the policy recommendations in the report.

Approximately 4,000 people are diagnosed with sarcoma each year in France. In Italy, the numbers are higher, with an estimated 5,890 of new sarcoma cases annually.

**CONNECTING SARCOMA PATIENTS WITH SPECIALISTS**

According to experts, the existence of reference centers in every country is a key aspect for the effective management of sarcomas.

In France, there are 28 such centers. The French Clinical Reference Network for soft tissue and visceral sarcomas (NetSarc) has proven successful, as patients treated by surgeons and oncologists within the network have seen better outcomes than those treated outside the network.

However, France lacks adequate numbers of trained specialised sarcoma surgeons. As a consequence, many patients are treated by generalists who are not experts in the field. According to the report, the country is currently considering developing a national network for sarcoma surgery with the ultimate

Continued on Page 13
goal of creating a “sarcoma surgery community”. Due to the complexity of sarcomas, experts recommend that patients seek a second professional opinion. Over 90% of patients have benefitted from second readings of their pathology reports by either the French Sarcoma Pathological Reference Network (RRePS) or the French Reference Network for bone sarcoma and rare bone tumours (ResOs).

Estelle Lecointe, who is President of InfoSarcomes one of the leading patient organisations in France for sarcomas, told Euractiv.com that access to diagnosis definitely remained the biggest challenge in France due to the rarity of these tumors. This logically leads to a lack of knowledge in non-specialist doctors and general practitioners.

“Patients are often misdiagnosed with other more common or benign diseases and some patients often have to wait for weeks or months to get the right diagnosis,” she said.

The Italian Rare Cancer Network, for its part, helps to connect sarcoma reference centres but is based on voluntary participation only and lacks formal accreditation.

However, there are ongoing efforts to establish quality indicators, and around ten sarcoma reference centres in Italy were chosen to be part of the European Reference Network (ERN) for sarcoma.

“Patients affected by rare cancers face the main problem of finding the right place to go for treatment in the initial phase of the disease,” said Dr Alessandro Gronchi, a sarcoma expert at the National Institute of Tumors (Istituto Nazionale dei Tumori) in Milan.

“It still happens a lot that patients are initially treated in the community where there is no specific expertise and referred afterward,” he added.

**NO FORMAL SARCOMA TRAINING**

While there is no formal training on rare cancers within the general medical curriculum or oncology training in either country, both have implemented some kind of specialised sarcoma training.

In Italy, there is a specialised surgical training programme for sarcomas at the European School of Soft Tissue Sarcomas. This is considered important by experts because surgeons are often the first place sarcoma patients go to seek care in Italy.

In France, an e-learning programme was set up by the Gustave Roussy Institute to better educate physicians and surgeons on sarcomas.

There are also ongoing discussions to include training on rare cancers in the future general medical school curriculum.

Lecointe explained that the second challenge for France was the “inappropriate surgery” as many patients are often diagnosed in non-specialist centers, where the first reaction is to remove the tumour (often confused with a cyst) without any prior biopsy and therefore without using the appropriate surgical techniques.

“This wrong initial management is unfortunately highly deleterious for the patients as the quality of the initial surgery will influence the overall outcome,” the expert noted.

**MULTIDISCIPLINARY TEAMS**

Experts recommend sarcoma patients be cared for by a multidisciplinary team (MDT), a group of several healthcare providers from a variety of disciplines, following a specific care plan.

In France, guidelines for sarcoma MDTs are produced and disseminated by NetSarc. There is no national definition of the minimum health care professionals who should make up an MDT, but patients do receive a personalised care plan.

In Italy, MDTs are neither mandatory nor monitored, but standards and guidelines are being incorporated into accreditation criteria for reference centres in the Italian Rare Cancer Network. Beginning in 2017, the organisation of MDTs is required for centres to be part of the European Reference Network for sarcoma.

**THE STATE OF SARCOMA RESEARCH**

Investing in sarcoma research is another tough challenge, considering the low numbers of patients and the lack of business interest.

In spite of this, France has become a leader in sarcoma research in international cancer genome projects: since 2013, 142 rare cancer studies have been carried out or are ongoing.

Sarcoma research in Italy currently includes the collection of incidence, prevalence, and survival data by the Italian National Association of Cancer Registries (AIRTUM), but the registry covers only 50% of the Italian population and does not always provide detailed information about pathology and patient outcomes.

“The research in the field of rare cancers suffers the same fragmentation of care,” Dr. Gronchi said.

“And it is penalised by the lesser impact that research in rare cancer has in attracting grants, making high impact publications, getting visibility and so on and so forth,” Gronchi added, underlining that there are a number of centres dedicated to research in rare cancers and specifically in sarcomas.

“They are well known all over the world and have managed to do top notch activities, although with Continued on Page 14
limited resources, but with a lot of enthusiasm,” he emphasised.

Because of this gap, the policy checklist calls for “prospective hospital-based data on the diagnosis, management, and outcomes of sarcoma patients”. Only two centres, in Milan and Bologna, currently collect this type of data.

The report also highlighted the significant research efforts being made for pediatric sarcomas in the country.

ACCESSING NEW TREATMENTS FOR RARE CANCERS

Another prevalent issue for the EU is the use of orphan drugs to treat rare cancers like sarcoma.

However, there are ongoing efforts to improve drug access for rare cancer patients.

The Italian Sarcoma Group and the Association of Italian Medical Oncologists (AIOM) both have lists of sarcoma trials taking place in Italy.

In France, the Autorisation Temporaire d’Utilisation, a programme that provides early access pathways, exists for life-threatening conditions but has not yet been used for sarcoma medicines.

The NetSarc-ResOs network allows access to national clinical trials but it is limited for patients not treated in a sarcoma reference centre.
European red tape should not be an obstacle to effective healthcare. For patients with diseases like sarcoma, waiting means suffering and often avoidable death, writes Marlene Mizzi.

Marlene Mizzi is a Maltese MEP from the Group of the Progressive Alliance of Socialists and Democrats (S&D group). Last week (6 February), she hosted an event in the European Parliament on the European policy response to rare cancers and particularly the case of sarcoma.

Last week, I hosted a policy debate on sarcoma – a form of rare cancer. Rare cancers have been given considerable attention within the EU lately. The Joint Action on Rare Cancers was launched last November, resulting in a dedicated European Reference Network (ERN) on Rare Adult Solid Tumours (ERN EURACAN). These developments are evidence that we are making concrete steps, at the European level, to try to address some of the considerable challenges faced by patients affected by these conditions. They also are excellent examples of what can be achieved when we gather experts across the medical, patient, policy and research world to develop focused policies with clear goals in mind.

Sarcomas are an important family of rare cancers and there are 27,908 new cases diagnosed every year in Europe. What is striking about sarcomas is their heterogeneity: they can affect any part of the body, and occur in approximately 70 different subtypes. This heterogeneity means...
that in practice, most doctors will not see certain types of sarcomas over the course of their career – and certainly not their medical training. As a result, like many patients with rare cancers (and indeed, rare diseases generally), sarcoma patients often live through late or misdiagnosis, difficult access to specialist care, and limited treatment options. More than 50% of patients with sarcomas are diagnosed in the advanced stage of disease, leading to poor prognosis. Given these statistics, it is no wonder sarcoma patients report some of the poorest experiences of care for any cancer type.

What struck me during our debate last week was the two sides to the sarcoma story that were presented. On one hand, there is huge progress in sarcoma policy, research, and models of care. Professor Paolo Casali, from Rare Cancers Europe and the Joint Action on Rare Cancers spoke about the fact that the ERNs, for example, should help achieve the multidisciplinary specialist model of high-quality care for sarcoma patients that we all aspire to. ERNs should also make it possible for patients with sarcoma who cannot find a specialist in their country to access highly skilled specialist care through their treating physician’s link with the ERN network. In other words, specialist know-how would travel, without the patient having to.

At the same time, Dr Peter Naredi, president of the European CanCer Organisation (ECCO) spoke about the fact that the ERNs, for example, should help achieve the multidisciplinary specialist model of high-quality care for sarcoma patients that we all aspire to. ERNs should also make it possible for patients with sarcoma who cannot find a specialist in their country to access highly skilled specialist care through their treating physician’s link with the ERN network. In other words, specialist know-how would travel, without the patient having to.

Notwithstanding these significant advances, Estelle Lecointe, a sarcoma patient herself and president of the Sarcoma Patients EuroNet Association (SPAEN), presented a more sobering view of the reality facing sarcoma patients today. Ms Lecointe said that, on a daily basis, the telephones at SPAEN are ringing off the hook with calls from patients who cannot find specialist care, or treatment, in their home country, and are desperately searching for ways to access it. What’s more, despite the Cross-Border Directive, patients are often not reimbursed for their care if they get treated abroad – or they only receive what their health care system would have paid locally, leaving them to fund the (often significant) difference out-of-pocket. This is not just a problem in some countries where gaps in specialist care are known to occur, mostly Eastern Europe. For example, in findings contained in the Sarcoma Policy Checklist, a report which was launched at the debate, it was shown that in Spain, centres of expertise are limited to big cities, and patients may not always receive reimbursement from their own region to be treated in another region.

These contrasting views illustrated to me, as a Member of Parliament, the gap between policy and implementation that sometimes, as EU-level politicians, we either fail to address or are not necessarily aware of. Yes, the EU has limited competencies over health care matters when it comes to the financing and delivery of care; but this is not a justification for us not thinking through, or ensuring that we evaluate, whether the policies or networks that we put in place at an EU-level are truly achieving what they should be doing: making a difference to patient lives.

I firmly believe that matters related to health should supersede European bureaucracy – and that sometimes, the onerous processes we have at the EU level can be an impediment to making things better for our citizens. Yet for sarcoma patients, waiting is simply not an option. Waiting means suffering and often avoidable death.

That is why I believe that if we work together as one community, we will be able to address all issues facing sarcoma, and rare cancer patients in Europe. The sarcoma policy debate showcased the willingness of all stakeholders to work together to try and address these issues.