

MICHEL RAGUET



MICHEL RAGUET IS VICE-PRESIDENT OF HAEI, the International Patients Organization for C1 Inhibitor Deficiencies. He is also the president of AMSAO, the French Association for patients suffering from angioedema due to C1 Inhibitor deficiency and is father to three children with this condition.

Can you tell us more about angioedema due to C1 Inhibitor deficiency?

Angioedema due to C1 inhibitor deficiency can be either hereditary or acquired and manifests as spontaneous edema (swelling) of the skin, the face, the extremities and, most worryingly, on the intestinal and laryngeal mucous membrane. Laryngeal edema, if untreated, can cause death by asphyxiation.

Three different types of angioedema exist and the most common (type 1) can be diagnosed by testing levels of C1 inhibitor, a protein found in human plasma. C1 inhibitor levels are extremely low in people affected by type 1 angioedema, which is the most common form.

During these last few years, two very effective treatments for angioedema have become commercialized. The first, a C1 Inhibitor concentrate (the missing blood protein) that is administered intravenously, is a plasma-derived product used to treat angioedema attacks.



The founding members of HAEI meeting in Grenoble, France. President Anthony Castaldo is second from the right, Michel Raguet is at the rear.

This treatment stops the development of the edema within a few hours, and prevents it from recurring during the days that follow. A second form of treatment is a medicine that blocks the activity of bradykinin, a vasodilator substance naturally present in human blood, but at higher levels in people with angioedema. It helps efficiently in blocking the onset of angioedema attacks.

When patients are properly diagnosed and treated, their quality of life is very good, and it is possible for them to forget about the disease almost completely. Unfortunately, this is not the case for all patients, and especially not those who have not yet been diagnosed. What has been noticeable for some time is that patients are starting to have a good knowledge of the different treatment options, as well as an increased dialogue with their physicians, in order to find the most suitable treatment for them.

Can you tell us more about HAEI and its work?

HAEI is an umbrella organization that represents 13 countries with active patients' organizations. It was founded in 2004, after several meetings between national association representatives. At the time, it seemed natural to gather various national patient organizations together as they pursued the same objectives—e.g., increased awareness and improved access to safe and efficient treatment. The advantage of an umbrella organization is better liaison with various stakeholders such as physicians, research centers, the industry, health authorities and so on.

Unfortunately, due to a lack of coordination, the association did not grow and progress as we had originally wished. However, this should change in future months, thanks to the support of the industry; our President, Anthony J. Castaldo; and our new Executive Director, Henrik Boysen, who will take things in hand to accelerate the projects' pace.

One of HAEI's main priorities is to inform patients about the range of treatments available to them. The chief difficulty is that the level of access to treatments differs from one country to another, according to each country's medical infrastructure. Another priority for HAEI is to help countries with no patient organizations to create their own. This process generally happens with the help of patients and physicians and, once done, HAEI provides guidance to newly-founded associations on how to improve access to treatment.



When did your work with AMSAO start?

I created AMSAO in 1997, with the help of some of my family members who were equally concerned with this disease. Originally, our main priority was to get access to information about the disease, the available treatments, the location of specialized physicians, the state of the research and greater understanding of the disease in general. At that time, finding this kind of information was hard, even for the patients themselves. The main problem was a lack of expertise among health care professionals in France.

Today, with the support of specialized health care professionals, AMSAO provides information to patients regarding their treatment options, as well as advice on how to tackle the disease and information regarding the state of research at the European level on the identification of complex mechanisms developing angioedema. These are patients' main priorities, especially finding a personalized treatment in order to get the best quality of life despite the disease. This is done through specialized reference centers.

In the past years the European Union and subsequently national governments in Europe have been implementing new policies to increase rare disease diagnoses and treatment.

What was the impact of these policies for patients and for patients' organizations?

European Union policies have motivated Member States to address the rare diseases issue in Europe. In France, for example, this effort resulted in the creation of a (specialized) medical reference center in Grenoble. This center brings together a wide variety of medical experts, such as genetic scientists, clinicians, biologists, etc. This multidisciplinary approach makes research progress faster. Moreover, the center is very active at the European level by sharing its research advances and monitoring other countries' progress. Following the creation of the reference center in Grenoble, seven branches have been established in France, creating a network across the country. These centers work closely with AMSAO, in order to diffuse information regarding scientific advances efficiently and effectively to patients.

What has been your proudest achievement in your work?

Through AMSAO's work, my proudest achievement was to make C1 Inhibitor available to ambulatory patients in France. Traditionally, patients needed to go to a hospital to get the drug administered and, at the time of AMSAO's creation, most hospitals did not have this medicine available. This proved to be a relief for patients as they could access the treatment whenever needed.

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