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Rare Juvenile Primary Systemic Vasculitis

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2. DIAGNOSIS AND THERAPY

2.1 What are the types of vasculitis? How is vasculitis classified?

Vasculitis classification in children is based on the size of the blood vessel involved. Large vessel vasculitis, like Takayasu arteritis, affects the aorta and its major branches. Medium vessel vasculitis typically affects arteries supplying the kidneys, bowels, brain or heart (e.g. polyarteritis nodosa, Kawasaki disease). Small vessel disease involves smaller blood vessels including capillaries (e.g. Henoch-Schönlein purpura, granulomatosis with polyangiitis or GPA, eosinophilic granulomatosis with polyangiitis or EGPA, previously referred to as Churg-Strauss syndrome), cutaneous leukocytoclastic vasculitis, microscopic polyangiitis).

2.2 What are the main symptoms?

Disease symptoms vary according to the overall number of inflamed blood vessels (widespread or just a few sites) and their location (vital organs like brain or heart versus skin or muscle) as well as the degree of blood supply compromise. This can vary from a transient minor decrease of blood flow to complete occlusion with subsequent changes to the unsupplied tissue caused by the lack of oxygen and nutrient supply. This can eventually lead to tissue damage with subsequent scarring. The extent of tissue damage indicates the degree of tissue or organ dysfunction. Typical symptoms are described under the individual disease sections below.

2.3 How is it diagnosed?

Diagnosis of vasculitis can be challenging. The symptoms resemble other various, more common paediatric illnesses. The diagnosis is based on an expert evaluation of clinical symptoms, together with the results of blood and urine tests and imaging studies (e.g. ultrasonography, X-rays, CT and MRI scans, angiography). Where appropriate, diagnosis is confirmed by biopsies taken from the involved and most accessible tissues or organs. Because it is rare, it is often necessary to refer the child to a centre where paediatric rheumatology is available, as well as other paediatric subspecialties and imaging experts.

2.4 Can it be treated?

Yes, today vasculitis can be treated, although some more complicated cases offer a real challenge. In the majority of properly treated patients, disease control (remission) can be achieved.

2.5 What are the treatments?

The treatment for primary chronic vasculitis is long-term and complex. Its main goal is to get the disease under control as soon as possible (induction therapy) and to maintain the control long-term (maintenance therapy), while avoiding drug side effects where possible. Treatments are chosen on a strictly individual basis according to the patient´s age and the disease severity.

In combination with immunosuppressive drugs, such as cyclophosphamide, and corticosteroids have proven to be most effective in inducing disease remission.

Drugs regularly used in maintenance therapy include: azathioprine, methotrexate, mycophenolate mofetil and low-dose prednisone. Various other drugs can be used to suppress the activated immune system and fight inflammation. They are chosen on strictly individual basis, usually when other common drugs have failed. They include the newest biological agents (e.g. TNF inhibitors and rituximab), colchicine and (less commonly) thalidomide.

For any patient requiring long-term corticosteroid therapy, osteoporosis should be prevented by sufficient calcium and vitamin D intake. Drugs

that affect blood clotting may be prescribed (e.g. low-dose aspirin and/or anticoagulants) and, in the event of raised blood pressure, blood pressure lowering agents are used.
Physiotherapy may be needed to improve musculoskeletal function, while psychological and social support for the patient and the family helps them to cope with the stress and strains of a chronic disease.

2.6 What about unconventional/complementary therapies?

There are many complementary and alternative therapies available and this can be confusing for patients and their families. Think carefully about the risks and benefits of trying these therapies as there is little proven benefit and they can be costly both in terms of time, burden to the child and money. If you want to explore complementary and alternative therapies, it is wise to discuss these options with your paediatric rheumatologist. Some therapies can adversely interact with conventional medications. Most doctors will not be opposed to complementary therapies, provided you follow medical advice. It is very important not to stop taking your prescribed medications. When medications such as corticosteroids are needed to keep the disease under control, it can be very dangerous to stop taking them if the disease is still active. Please discuss medication concerns with your child’s doctor.

2.7 Check-ups

The main purpose of regular follow-up is to evaluate the activity of the disease and the efficacy and possible side effects of the treatment, in order to achieve maximum benefit for your child. The frequency and type of follow-up visits depend on the type and severity of the disease, as well as on the drugs used. In the early stage of the disease, outpatient visits are typical and, in more complicated cases, inpatient admissions can be more frequent. These visits usually become less frequent as soon as disease control is achieved.
There are several ways to evaluate disease activity in vasculitis. You will be asked to report any changes in your child’s condition and in some cases to follow his/her urine dip-stick tests or blood pressure measurements. Detailed clinical examination together with the analysis of your child’s complaints form an important part of the evaluation of

disease activity. Blood and urine tests are performed to detect activity of inflammation, changes in organ functions and potential drug side effects. Based on individual internal organ involvement, various other investigations might be performed by different specialists and imaging studies may be required.

2.8 How long will the disease last?

Rare primary vasculitides are long-term, sometimes life-long diseases. They can start as an acute, often severe or even life-threatening condition, and subsequently evolve into a more chronic low-grade disease.

2.9 What is the long-term evolution (prognosis) of the disease?

Prognosis of rare primary vasculitides is highly individual. It depends not only on the type and extent of vessel involvement and the organ involved, but also on the interval between disease onset and the start of treatment as well as on the individual response to therapy. The risk of organ damage is related to the duration of active disease. Damage to the vital organs can have life-long consequences. With proper treatment, clinical remission is often achieved within the first 6-12 months. The remission can be life-long but long-term maintenance therapy is often needed. Periods of disease remission may be interrupted by disease relapses requiring more intensive therapy. Untreated disease has relatively high risk of death. Because the disease is rare, exact data on long-term disease evolution and mortality are scarce.