THE MYOSITIS ASSOCIATION

JM COMPANION

For children, families and friends affected by juvenile myositis

Juvenile myositis: An update

Better and more individualized treatments for juvenile dermatomyositis, plus better ways to predict and measure the effectiveness of therapy, have greatly changed the outcome for today's families. Although patients with adult forms of myositis have appreciable mortality (about 10%) in some cases -- and progressive disability in others -- outcomes are more often excellent in children. "We have not had a single death from JDM in 30 years," said Dr. Brian Feldman, a pediatric rheumatologist at the Hospital for Sick Children in Toronto and member of TMA's medical advisory board.

Specialist collaboration, new drugs and a better understanding of JDM are improving the lives of patients.

In earlier decades, almost one-third of juvenile myositis patients died and one-third sustained damage that remained with them throughout their lives. Collaboration between specialties, new drugs, and a better understanding of how to use older drugs has resulted in a great improvement, not only in saving lives, but in increasing the quality of life for juvenile myositis patients.

In a recent study led by TMA medical advisory board member, Dr. Adam Huber, *Update on the assessment of children with an idiopathic inflammatory myopathy*, that included several major North American hospitals, outcomes were also optimistic. In one study of 65 juvenile myositis patients followed for seven years, there was only one mortality, and the overall news was good.

The children reported excellence in social, academic and work-related achievements. Patients also expressed relative satisfaction with their lives.

The study found that more than a third of the young patients were still on medication for the disease seven years later, including 15% who remained on prednisone. Other medications were methotrexate, cyclosporine and hydroxychloroqui-

Continued on page 2.

Member dues eliminated

The board of directors of The Myositis Association made a milestone change in the operations of TMA this year with the elimination of dues. For some patients, even the modest amount of $15 or $35 was a deterrent to joining the Association.

In support of TMA's mission to serve all those with myositis, the Board of Directors decided that elimination of dues was the right thing to do.

TMA continues to welcome donations from those served by the Association to help support its work. However, a financial contribution is now no longer required to receive services previously only available to members – the quarterly newsletters, online chats with medical experts, email updates regarding research and clinical trials, etc.

Additionally, the TMA board undertook a strategic planning process in 2011 to focus the organization's resources on areas critical to serving the mission of TMA and providing cost-effective service to patients and their caregivers. Results of the strategic planning process will help guide TMA's activities in 2012.

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JM Update, cont'd from cover

nine (Plaquenil) and combinations of these and prednisone.

Many things have contributed to the changing face of juvenile myositis:

The childhood health assessment questionnaire

This was originally developed for evaluating disability in children with arthritis, and quickly became used by pediatricians as a valid and responsive tool for juvenile myositis patients. In one study, the questionnaire was tested in a population of 37 children with JM, and was shown to correlate well with disease activity, to be reliable even in patients who did not have a change in muscle strength that could be observed in the office, and to be responsive to clinical change after treatment.

The childhood myositis assessment scale

The 14-point scale is an occupational measure of muscle function. It uses a series of activities chosen by a group of experts in the field to measure patient ability. This tool, along with the health assessment questionnaire, makes it possible for physicians to predict outcomes in children with juvenile myositis. Members of TMA's medical advisory board who are pediatric rheumatologists took a leading role in the collaboration that produced this tool. Most clinicians who work with children now use the scale.

Dr. B. Anne Eberhard, a pediatric rheumatologist at the Albert Einstein College of Medicine in New York, talked about the scale in an address to pediatric rheumatologists at an annual meeting of rheumatologists. She explained that the scale can compare, from one visit to another, how well a child can raise his head or leg, go from a supine to sitting position, rise from a chair, step on a stool, and nine other maneuvers. Pediatric rheumatologists as well as family pediatricians welcomed the scale because it allows them to check on the progress of their patients in the office, without always having to use elaborate and sometimes intrusive laboratory tests.

The scale is also useful for researchers as a way to measure muscle strength, physical function and endurance when evaluating how a patient responds to a drug or other intervention after treatment.

Clues at your fingertips

A growing number of research studies describe another simple, non-intrusive way that clinicians can check on disease activity in their young patients. The underlying disease in JM is inflammation of the capillaries, the extremely small blood vessels that deliver blood throughout the body. It's easy for doctors to see the capillaries at the nailfolds – the place where the skin joins the nail. In fact, parents often notice inflammation there without magnification. It's now increasingly recognized that examining the nailfold is a simple, noninvasive means of monitoring disease activity in children with JDM. Your physician might use a stereomicroscope or a simple magnifying glass to observe these capillaries.

The changes the doctor is looking for are a reduced number of capillaries, signs of hemorrhage, and branching that looks abnormal. In the past few years, many researchers and physicians have found that simply counting the capillaries at the fingertip edge of the nailfold can strongly predict overall disease activity, muscle strength and function and increase or decrease in the skin rash that accompanies the disease. In one recent study, capillary density in the nailfold strongly reflected disease activity in 42 children. As the disease activity increased, capillary density was reduced.

Treatment improves

Rethinking "pulse" prednisone

A survey of more than 170 pediatric rheumatologists in North America showed that 15% of them start

patients with newly-diagnosed typical juvenile dermatomyositis on oral prednisone therapy compared to the 85% who start with relatively high-dose intravenous methylprednisolone. This IV therapy became a traditional practice with children because of the belief that it's better tolerated and more effective. According to recent research reported by Dr. Brian Feldman and Dr. Angelo Ravelli, both pediatric rheumatologists, this may not be accurate.

In a study of 42 patients treated aggressively with intravenous methylprednisolone, and 42 patients treated with lower daily doses of oral prednisone, the study found that outcomes were similar for both treatments. This determination was made by following up with the patients after three years of treatment. The 42 patients were closely matched by a scoring system, to rule out differences in the disease characteristics that may have contributed to initial treatment choice. Since oral prednisone has fewer side effects, the study suggests that it may be the better choice.

Supplementing treatments with daily vitamins is recommended.

Increasing use of methotrexate with prednisone

This combination, also increasingly used to treat adult myositis, has been shown to reduce the amount and length of prednisone treatment by halving the exposure and total cumulative dose, reducing the well-known prednisone side effects while being just as effective as the higher dose.

Patients treated with methotrexate were tapered off of prednisone by having their dose lowered by 10% every two weeks, compared with 10% per month in control patients who were treated with only prednisone. According to the study, published in Arthritis, Rheumatism, those in the methotrexate group had less weight gain and better growth, compared with control patients.

IVIG for some cases

Studies by the Hospital for Sick Children and the Cleveland Clinic show that IVIG is generally safe for children. In one study of 78 patients, including 30 children who either did not respond to prednisone, who could not be weaned from prednisone, or whose disease worsened despite conventional treatment, those treated with IVIG improved more—and more quickly—than those who were not treated with IVIG. Another study chose several children to treat from the outset with methotrexate and IVIG alone, and all children improved. Because of the expense of IVIG, it is not considered a first-line treatment for juvenile dermatomyositis.

Greater focus on protecting bones

Because it is both inexpensive and effective at controlling inflammation (see above) prednisone is widely used to treat patients with juvenile myositis. Many of these patients are already at risk of osteoporosis because the inflammatory nature of their disease also causes bone loss.

As yet, there are no evidence-based guidelines for the prevention and treatment of prednisone-induced osteoporosis in children and adolescents. General measures include using the lowest effective dose for the shortest period of time, and considering alternate therapies, calcium and vitamin D supplementation, weight-bearing exercise, and proper nutrition.

The general deficiency of vitamin D in the general population is being recognized. Children with JM, who must be protected from direct sunlight and often take drugs that contribute to bone loss, are believed to be at even greater risk than other children. Although there are no specific guidelines for children with autoimmune disease, the Mayo Clinic suggests that vitamin D may have some protective properties against immune defects as well as bone thinning in children.

Since 2000, when widespread vitamin D deficiencies were first being reported, discrepancies have arisen regarding the benefits of vitamin D and how much to take. In 2008, the American Academy of Pediatrics increased its recommended daily intake of vitamin D in infants, children, and adolescents to 400 IU.

The new recommended daily allowance, set last year, is based on age, as follows: for those 1-70 years of age, 600 IU daily; for infants aged 0-12 months, the upper level intake is 1,000 IU daily. Other research confirms these recommendations. Make sure to check with your child's physician about any medications, including vitamin supplements.

Use of imaging to identify and track inflammation

Since there is no specific blood test for JM, doctors use a variety of information to make a diagnosis. The most characteristic finding in the lab tests is increased level of one or more muscle related enzymes.

Other tests, like muscle biopsy and EMGs, can be frightening and uncomfortable for children, so more physi-

Continued on page 5

Make exercise part of the JM prescription

We've known for some time that appropriate exercise is not harmful for children with JM. Now there's growing evidence that encouraging regular physical activity is one of the most positive interventions a family can make for their child. In an article titled *Evidence for prescribing exercise as treatment in pediatric rheumatic diseases*, Dr. Bruno Gualanoa, a Brazilian rheumatologist, and other physicians and sports medicine experts make a strong case. The article, recently published in "Autoimmunity Reviews," recommends that physical training be accepted as a treatment of pediatric rheumatic diseases.

"Remarkably," the authors write, "recent evidence suggests that exercise may have direct effects on the pathogenesis of autoimmune diseases by attenuating chronic low-grade systemic inflammation."

First, Dr. Gualanoa *et al* examine the lack of regular exercise or physical inactivity as a contributing cause in all chronic diseases, citing the already-established view that regular exercise offers protection against all kinds of sickness and untimely death.

Further, they say, a body of evidence from randomized intervention studies shows that physical training is effective as a treatment in children, adolescents and adults with various chronic conditions, such as type 2 diabetes and cardiovascular diseases, which are strongly associated with chronic systemic inflammation.

Modern science has long since challenged the old-fashioned clinical treatment of active rheumatic diseases with bed rest. One of the earliest studies to debunk this therapy demonstrated that patients with rheumatoid arthritis who underwent an exercise training program had more significant improvements regarding hand pain, joint tenderness, and activities of daily living score when compared to patients who did not exercise.

Gualanoa and his colleagues would like to see a shift from the paradigm of bed-rest treatment in the childhood diseases toward the compulsory recommendation of exercise training for rheumatic patients. Adding to their concern, the authors note, is the growing realization that children and adolescents, regardless of their health, have become more physically inactive. As a consequence, the authors note, the incidence of early-onset chronic diseases has increased dramatically.

"Moreover," they say, "the lack of physical activity may also aggravate muscle weakness, atrophy, muscle dysfunction, chronic fatigue, motor-control disturbances, mood disorders, bone resorption, dyslipidemia, obesity, arterial hypertension and insulin resistance in pediatric rheumatic disease patients."

The authors recognize some of the hardships that prevent children with rheumatic disease from exercising – pain and stiffness, contractures, fatigue and weakness. These obstacles set up a vicious cycle that ultimately leads to physical de-conditioning, symptom aggravation and poor quality of life. In this context, exercise training emerges as the most evident therapeutic tool for stalling the downward spiral, the authors contend.

The study examines the JM population in particular. "Patients with JM often experience strong exercise intolerance. Given that cardiac or pulmonary involvement is uncommon, the major contributor to the impaired exercise capacity is the pathologic change in muscle tissue. There are several explanations for the significant impairment in exercise capacity in patients with JM: the increased concentration of intramuscular cytokines, the systemic inflammation process, the inflammation of the capillaries in the muscle, the result of reduced activity, and the effect of glucocorticoid treatment on body mass gain and protein breakdown.

"Moreover, abnormal high-energy phosphate metabolism (e.g., lower muscle phosphocreatine content), as measured by magnetic resonance spectroscopy, suggests that children with JM may have an impaired muscle oxidative capacity. Theoretically, exercise training might benefit JM patients by reducing systemic inflammation and vascular reactivity, improving prednisone-induced deleterious effects, increasing muscle mass and neuromuscular function, reducing fat accumulation, and improving oxidative capacity and neovascularization."

A natural anti-inflammatory

In addition to the benefits of exercise training in preventing further functional loss, the authors propose that exercise might be especially helpful in reducing the inflammation of juvenile myositis. Drawing from studies of healthy people, they found ample evidence of a positive association between physical inactivity and low-grade systemic inflammation. Other studies indicate that exercise training promotes a reduction in C-reactive protein concentration, further suggesting that physical activity may suppress systemic low-grade inflammation.

Thus, they speculate, exercise training can reduce chronic inflammation in pediatric rheumatic patients, possibly reducing either the number or the dose of immunosuppressive drugs.

Continued, next page

Further remarks

The authors lament the time and energy going into research that aims to develop a pill or series of pills to counteract the side effects of inactivity and imitate the beneficial results of a physical training program. This won't work, they say: "All health benefits of exercise are too complex to be replaced by a single pill. Since exercise produces adaptations in multiple organs, exercise has natural, built-in specificity that is unmatched by the therapeutic limitations of drugs. Furthermore, it is unlikely that any drug can be more cost-effective to the health care system and safer than natural physical activity itself."

They contend that exercise may be better primary medicine than any current drug for several groups, including pediatric rheumatic patients. They call for efforts to increase studies of the efficacy of exercise in this population. A Dutch study presented to a pediatric rheumatologist working group demonstrated that many of the factors that seemed to prevent children from exercising during remission from JM actually improved once the children did prescribed exercise.

In Australia, pediatric rheumatologists routinely recommend exercise for their juvenile patients, according to the Myositis Association of Australia, advising them to do what they can, according to their level of disease activity:

- Aerobic exercise assists patients by increasing the function of the cardiovascular system as well as the endurance of the muscles. This can improve the capacity for physical activity, work and activities of daily living and will also improve measures of fatigue. Aerobic exercise may include walking, swimming or riding stationary bikes.

- Strength training, properly prescribed, will increase muscle mass in patients with myositis and will also improve the challenges of balance and gait. Strength training programs can be individually tailored to the patients needs and can include activities such as free weights.

- Balance training can maintain and improve balance for patients with myositis whose balance may be affected by loss of muscle mass or pain. Yoga, tai chi, karate, snowboarding and skiing are all good balancing exercises.

RecognizedResearcher

Dr. Rider, physician researcher of the year

Dr. Lisa Rider, founding member of TMA's medical advisory board, was chosen from 900 medical officers to receive the physician researcher of the year award for 2011 by the US Public Health Service. The award honored her basic and clinical research into juvenile myositis and praised her achievements as being in the highest tradition of the Health Service. The award also mentioned Rider's role in a recent landmark study, published in JAMA, on the classification of myositis types.

Dr. Rider, who is deputy chief of the Environmental Autoimmunity Group at NIH. acknowledged the help of Dr. Fred Miller, who heads the group. Dr. Miller is also a founding member of TMA's medical advisory board.

Find Dr. Riders research at www.myositis.org.

JM Update, cont'd from page 3

cians are using magnetic resource imaging (MRI) as a way to view the muscles. MRI does not involve X-ray exposure. MRI can be used to either help make the diagnosis of JM or to find inflamed muscles most suitable for muscle biopsy, so that multiple biopsies will not be needed. On follow-up visits, markers of inflammatory change in JM can be observed on MRI in a reliable fashion and have been used to make a practical and objective scoring system.

TMA resources for JM

The Myositis Association has many resources online at www.myositis.org. Some of the most helpful follow.

Patients talk about their experience with prednisone: http://www.myositis.org/pdf/JMBookResources/7.3.pdf

Dr. Ann Reed presents a comprehensive overview of JM, including treatment and research: http://www.myositis.org/pdf/CONF08/2008_Reed_JM.pdf

Handout for teachers: http://www.myositis.org/pdf/Juvenile/JMBrochure_TeachersEdition.pdf

JDMS: new developments in pathogenesis, assessment and treatment: http://www.myositis.org/pdf/New%20development%20in%20pathogenesis,%20assessment%20and%20treatment.pdf

Comprehensive review of JM by Dr. Adam Huber of TMA's medical advisory board: http://www.myositis.org/pdf/Huber%20JM.pdf

Presentation by Dr. Daniel Lovell on new treatments and trials: http://www.myositis.org/pdf/CONF06/2006AC_medpanel_lovell.pdf

Treatment options for JM: http://www.myositis.org/pdf/JMBookResources/7.1.pdf

Published JM Research: http://www.myositis.org/template/page.cfm?id=85

GET READY FOR SKI AND SNOWBOARD SEASON

Whether you're in a wheelchair or experiencing the muscle weakness of JM, there are national organizations designed to help you stay active. Exercise is important for everyone: for JM it is an important part of influencing the disease process as well as aiding in recovery (see page 3).

Disabled Sports is a nonprofit organization first established in 1967 to serve those injured in Vietnam. Now the agency serves all those with disabilities year-round, and you can find a great deal of information, as well as contact information for your area at www.dsusa.org.

Meanwhile, the organization is gearing up for the winter sports season. Winter is a great time to get outdoors, since the sun doesn't cause as many skin flares for JM patients. Some questions and answers about winter sports, courtesy of Disabled Sports, will help you get started.

I'd like to learn to ski, and eventually race. How do I get started?

There are many ski resorts throughout the country that offer programs for people with disabilities. Some adaptive programs are chapters of Disabled Sports USA and are part of a ski area's non-disabled ski school, while others are separately run non-profits. Visit the web site and click on the chapter listing tool to view the programs and locations close to you. Some programs have "Learn to Race" opportunities and others offer advanced level training.

What types of disability can participate?

People with all types of disabilities, including neuromuscular impairments, enjoy skiing. The instructors listed in the area programs have many years of experience and can answer questions directly concerning your unique situation. There are many adaptations that can be made to equipment and instruction to enable people to participate.

What equipment will I need?

Appropriate equipment is crucial for a successful experience. Some adaptations offered are outriggers, monoskis, and guides. Avoid purchasing your own equipment until you've tried out what's available for comfort and fit. Also bear in mind that as you become more skilled, new equipment may be required. For those ready to invest, a list of equipment resources can be found at http://www.dsusa.org/equipment/skiing.html.

Keep in mind that adaptive programs usually have a range of equipment available for rental. You should contact individual programs in advance for specific information.

Are there grants to help me participate?

Yes, there is financial assistance to help you learn to ski and race. Ask your local DSUSA chapter if they offer a scholarship program. For equipment and competition grants, contact the Challenged Athletes Foundation at http://www.challengedathletes.org. For young emerging athletes who want to eventually compete at the national level, Disabled Sports offers scholarships to attend the Hartford Ski Spectacular, one of the largest winter sports festivals for people with disabilities. Find out more at http://www.dsusa.org/DianaGolden/index.html.

How can I learn more about alpine ski racing?

The following DSUSA chapters have active adaptive race programs:

National Sports Center for the Disabled (Winter Park, CO), www.nscd.org.

National Ability Center (Park City, UT), www.discovernac.org.

Aspen Valley Ski Club (Aspen, CO), www.teamavsc.org.

Maine Handicapped Skiing (Newry, ME), www.skimhs.org.

Adaptive Sports Foundation (Windham, NY), www.adaptivesportsfoundation.org.

If there is not an adaptive race program near you, most U.S. Ski and Snowboard Association (USSA) clubs will welcome racers with disabilities. Reach out to your local club via their website, www.ussa.org, Also, consider attending the National Race Festival at The Hartford Ski Spectacular, located in Breckenridge, CO, to meet coaches, athletes, and find out more about what the commitment entails.

More great info! Be sure to check out "Transition Passport" on page 8.

JUST FOR ME

Fun facts and news for children affected by JM

Waiting for a blood test?

No one likes needles, but it's not so bad if you know what to expect. Find a preview at: http://kidshealth.org/kid/feel_better/things/video_bldtest.html.

A fingernail tale

Ever wonder why your doctor looks at your fingertips? It turns out that your nails tell your doctor a lot about your illness. Here's why.

JM affects your capillaries, tiny blood vessels that distribute blood throughout your body. The disease makes them swell up. This is what causes your rash and muscle weakness.

Capillaries are so tiny that dozens of them fit into a fingertip. Rather than just ending there, they form loops to return the blood to other parts of your body. That's one of the reasons that your fingertips have so much feeling. Because a lot of capillaries are located near your nails, where the skin touches your fingernail, your doctor can look there to see what is happening with them.

If you have extra pain, redness or swelling in your fingertips, be sure to tell your parents.

JM Friends

Most likely, you don't know anyone with JM, and you may feel lonely at times, as though no one understands what you're going through. You can find photos of people with JM, find out what their lives are like, put your own story and photo online, and ask to be put in touch with other JM children by going to www.myositis.org.

Click on "patients and families," then "kids," then "JM Friends."

To find a JM friend near you, email tma@myositis.org.

Snowflake Facts

How does snow form?

Snow crystals form in clouds when the temperature is colder than 32 degrees. Snowflakes are water droplets that freeze into tiny pieces of ice. As an ice crystal drops through the cloud it bumps and knocks others and they combine to become a snowflake. The air that the snowflake drops through has to be under freezing otherwise the snowflake will simply melt and turn into rain.

What do snowflakes look like?

Snowflakes always have six sides and are like a human fingerprint, because two snowflakes are ever exactly the same.

Snowman Fact!

The world's tallest snowman ever built was over 100 feet tall and was made by the people of Bethel, Maine in 1999. It took 2 weeks to build! The snowman, nicknamed Angus, was so big that he had car tires for his mouth and trees for arms!

The Myositis Association

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FreshOutLook

Not just for adults

Take the time to check out TMA's 2011 Annual Patient Conference coverage in the OutLook and at www.myositis.org. There are many helpful presentations, but keep in mind that adult and juvenile myositis differ in several very important ways:

- **Prevalence.** Dermatomyositis accounts for 90% of myositis cases in children but only 11%–14% in adults.
- **Cancer risk.** In children, cancer is not associated with myositis. In adults, up to 14% of patients will have associated malignancy.
- **Systemic manifestations.** These are uncommon in children, common in adults.
- **Cardiac disease.** Inflammation often contributes to heart disease in adults, almost never in children.
- **Calcinosis.** These deposits of calcified material are common in children, uncommon in adults.
- **Disease course.** Outcomes are better in children, and remission is usually permanent, although the disease is often chronic. In adults, the course is often progressive.

Do you have a "transition passport?"

A study of anonymous JM parents led by Dr. James Katz of George Washington University's Myositis Center showed that many parents of teenage JM patients did not believe their children were ready to transition to adult care.

Some of the questions you can ask yourself as you get ready to make the transition are:

- Do you see the doctor alone?
- Do you know the names and telephone numbers of the health care professionals you deal with?
- Do you arrange your own appointments?
- Do you arrange for refills of your prescriptions?
- Do you have a full copy of your medical records?

If you have answered "yes" to some of these questions, you're on your way. One good way to make sure you can deal with your medical care as an adult is to create a written transition plan with your parents and physicians.