THE MYOSITIS ASSOCIATION

JM COMPANION

For children, families and friends affected by juvenile myositis

Ask the doctor: treatment, prognosis, complications

Dr. Adam Huber is a pediatric rheumatologist at the IWK Health Centre and an associate professor of pediatrics at Dalhousie University in Halifax Nova Scotia. He did his pediatric rheumatology training at the Hospital for Sick Children, where he developed an interest in juvenile myositis while working with Dr. Brian Feldman. He is an active clinician, with a particular research interest in the assessment of disease activity and outcome in children with juvenile myositis. He answered questions from families of children with juvenile myositis in July, and portions of the discussion are excerpted below. For the full transcript, go to www.myositis.org.

TMA Member: Do rheumatologists have a better understanding of how to treat JDM now than they did 10 years ago? What is the more recent thinking?

Dr. Adam Huber: Ten years ago, we certainly used steroids for longer periods of time, and were less likely to use second-line agents (particularly methotrexate) than we are now. Many rheumatologists would now have children with myositis off steroids in 9 to 12 months (assuming a good response) and most would start methotrexate at the beginning of treatment. The hope is that outcomes are just as good, but with a reduction in steroid side effects. I think there is some early evidence that this is true, but probably not enough to be certain. There are some new medications (like rituximab), but their role and effectiveness remains to be proven.

TMA Member: How effective is IVIG in treating JDM?

Dr. Adam Huber: In my experience, and that of many of my colleagues, IVIG can be an extremely effective medication. I tend to use it in patients who are responding slowly at the start of therapy, or look as though there may be a fairly severe course. It can also be extremely effective for chronic skin disease. Finally, I will often use it as an alternative to increasing steroids when there is a flare of disease. The biggest problem with IVIG is access (both the need for IV access, and cost issues). For some children, headache or nausea can also be limiting.

TMA Member: There are often varying opinions from doctors, patients, and caregivers regarding dermatomyositis and cancer. These varying opinions make it difficult for the patients since some doctors do not screen enough and some may screen too much. I am concerned because of how difficult I have been to treat after a confirmed diagnosis of both DM and Sjogren's and a very strong family history of colon, stomach, and lung cancer in my family in every generation going back four generations. I am 56. I obviously have concerns for my children and grandchildren.

Dr. Adam Huber: The issue of cancer in myositis is quite clear in adults. There is an association. However, the strength of that association is probably not as strong as has been reported at some times. Up to 1/3 has been often quoted. However, more recent statistics have suggested that the actual percentage is less, likely closer to 10 percent.

The issue in pediatrics is considerably more complicated. There have been a very small number of case reports of children with myositis syndromes who have cancer. In fact, the numbers are so small that it is likely that there is not an association of childhood myositis with cancer. In general, children are not screened for cancer, beyond routine blood work. This is partly related to the low likelihood of there being a cancer to find, and partly that the types of cancers we see in children, like leukemia, are normally picked up on fairly routine blood work that we would do in any patient.

Continued on page 2.

Published by: TMA, Inc.
THE MYOSITIS ASSOCIATION
1737 King Street, Suite 600
Alexandria, VA 22314
P: 703.299.4850
P: 800.821.7356
F: 703.535.6752
Email: tma@myositis.org
Web: www.myositis.org
Executive Director: Bob Goldberg
Editor: Theresa Reynolds Curry

Q&A

Continued from cover

TMA Member: What are the risks that a child with chronic JDM will have long-term joint and muscle damage?

Dr. Adam Huber: The literature on predictors of permanent damage is somewhat limited. There are some factors that have been associated quite consistently--in particular late or delayed treatment and inadequate treatment (not enough or too brief). Some of this was described back in the 1980s.

The impact of new approaches (such as starting second-line agents like methotrexate at diagnosis) is unknown. There are some recent data suggesting features of the muscle biopsy can predict poor outcomes, but this is quite preliminary. Otherwise, it is quite difficult to predict the course, and therefore the outcomes, for children with myositis. However, in the next year or so, there will be some publications (both from the European group and the North American group) that may help to answer this question to a more satisfactory degree.

TMA Member: What percent of those with JDM develops calcinosis?

Dr. Adam Huber: This is a surprisingly difficult question. Some series have shown rates of calcinosis that were quite high (up to 40 percent). However, in other clinics, it is clear that calcinosis is quite uncommon. In our clinic, calcinosis appears to be quite rare. We do not understand the factors that lead to calcinosis particularly well. There are probably genetic factors as well as environmental ones. It has been suggested that chronic skin disease increases the risk of calcinosis, based on MRI studies. It is also likely that delayed or inadequate therapy increases the risk. However, the variation in calcinosis rates is not explained by differences in treatment. I am not sure if this is a very satisfying answer, but is probably the best we can do in 2010. As you can appreciate, the prevention of calcinosis is an important goal of myositis treatment, given that there is little effective therapy.

TMA Member: How soon after treatment begins should we expect to see improvement?

Dr. Adam Huber: It is difficult to make any concrete response to this question, although it certainly is a good question. We see some patients respond very rapidly (within one or two weeks), and others seem to take eight weeks or longer. I would certainly prefer to see some evidence of response (or at least an arrest of any deterioration) within four to six weeks and hopefully sooner. It is important to keep in mind that there is some good evidence from Dr. Pachman in Chicago that steroids by mouth may not be absorbed very well in children with active myositis, which could delay response. In that case, the use of IV steroids may be better.

TMA Member: Are there any new treatments for calcinosis? Do you recommend surgery for calcinosis and in your experience does it quickly grow back?

Dr. Adam Huber: I am fortunate in that our clinic. For whatever reason (I don't think it is my superior care!) we see very little calcinosis. At this point, I have not been convinced that there is any effective treatment for calcinosis. There have been many reports of a variety of medications, but none has been very convincing. Assessment of any therapy is complicated by the fact the calcinosis sometimes clears on its own, without intervention. I think there are definitely circumstances where surgery is useful. If the area is particularly vul-

nerable (on a joint) or causing dysfunction or pain. It is reported that the surgery can stimulate the calcinosis to grow back, although I have seen cases where this was not the case. I think it is very important to have the disease under good control when the surgery is done to minimize the risk of recurrence. Finally, the most important treatment for calcinosis is to prevent it in the first place.

TMA Member: My child has a very severe case and is on high doses of prednisone. He is bloated and hungry all the time. My doctor and I believe the prednisone is necessary at this point to save his life, and do not want to taper yet. Is there anything I can do to help modify the effects of prednisone? My child is six years old.

Dr. Adam Huber: This is a common problem, and not one that has any easy answers. Prednisone really is a double-edged sword. We need the positive effects on the disease, but really hate the side effects. There is not much that is hugely helpful. We have all our patients work with a dietician to minimize weight gain as best we can. A psychologist can be helpful to deal with some of the behavioral issues, and assist with strategies to deal with the sometimes overwhelming hunger (and help with issues around having a chronic illness, changes in appearance, etc). In the end, the best thing you and your doctor can do is to reduce the prednisone as quickly as is safe. I agree that the side effects can often be miserable, and may even feel worse than the disease.

TMA Member: I am an 18-year-old girl with JM and I am in remission after three years of treatment. I am still on 5 mg. of prednisone. Is this really a remission? What would happen if I stopped taking the prednisone?

Dr. Adam Huber: You raise a really interesting question. Definitions of terms like "remission" are not always consistent. I think the most commonly used definitions of remission now include "off medications". For that reason, I would hesitate to use the term remission for someone still on 5 mg of prednisone. However, you MIGHT be in a true remission. By this I mean it is impossible to know right now if you are doing well because your JDM has gone away (remission), or if it is because the little bit of steroid is controlling it very well. The only way to know is to continuing weaning the steroid, carefully and with regular evaluations. Suddenly stopping the steroid could be dangerous – both because of the possibility of a disease flare and because your body needs to time to start making its own steroid, and suddenly stopping steroid would not allow this to happen. This is certainly something you should discuss with your doctor.

TMA Member: What is it in a child's genetic background that makes him or her susceptible to JM? Have they found the genes responsible yet?

Dr. Adam Huber: At this point, we have some genes that are associated with myositis in children, although I think it is premature to say that we "know" the cause. There are some genes in a very important area called HLA that seem to increase or decrease the chance of developing myositis. There is also a gene polymorphism (a slight difference in the gene that is quite common in the population) in a gene called TNF-alpha which seems to increase the chances of developing JDM. There are probably many other genes that predispose to developing myositis – it is likely that several are needed, and probably some sort of trigger (an infection maybe) for a child to develop myositis.

TMA Member: I eventually want to have a child. Is this advisable? What should I watch out for? I am an 18 year old female in remission except for a very small dose of prednisone. Are my children more likely to have myositis?

Dr. Adam Huber: There is little evidence that your ability to have a child is affected by having myositis. It is possible that a flare could occur, as there are fairly major changes that occur in the immune system during pregnancy. From a safety point of view, you would be off medications and you would want to be carefully monitored during the pregnancy, both for your health and that of your baby. It is possible that certain medications could affect your ability to become pregnant (mainly if your disease was severe enough to require cyclophosphamide). Some medications that you may still be on could have bad effects on a baby (methotrexate for example). The likelihood of your children developing myositis or another autoimmune illness is probably slightly increased. However, this risk is still very small, and it remains much more likely that your children will not be affected.

TMA Member: We just had a JM Conference in Rochester, MN, and the kids all "looked" really well. When the children look well and are obviously doing pretty well, what are the challenges parents must face to keep them feeling and doing so well? Is diet involved?

Dr. Adam Huber: There are probably few recommendations that can be made to help them to stay that way. I do continue to recommend continued sun-protection, which is probably relevant to all of us anyway. Otherwise, continued healthy living is as good as we can do: regular exercise, healthy diet, and routine health monitoring (bone health, cholesterol etc). There are not specific activities or dietary factors etc that we know of that can prevent future flares.

TREATMENT UPDATE BY DR. ANN REED

Dr. Ann Reed discusses current thinking in myositis drugs, diet and exercise

Ann Reed, a professor of medicine at the Mayo Clinic in Rochester, Minnesota, teaches and practices in pediatric rheumatology, and is the author of many JM studies. She spoke at a day-long TMA seminar for juvenile myositis patients at the Clinic on July 17. The following is a summary of her presentation.

Prednisone

It's still the lifesaving, first-line treatment for JM, but physicians who see a lot of JM patients know it's crucial to taper prednisone (a steroid drug) as quickly as possible. "Prednisone itself causes a kind of myopathy," Dr. Reed said. "We know that it causes some of the muscle inflammation we see in children who have been on high doses for a long time."

This side effect enters into the discussion of how the prednisone should be delivered. Some physicians choose intravenous prednisone for their patients, Reed said, especially when a large dose is called for. "This is a big debate - who should get it and under what circumstances," she said, "but in the long run, there doesn't seem to be much of a difference in the outcome."

Methotrexate

In North America, physicians usually choose to start methotrexate either with the prednisone, or shortly after, to reduce the total amount of steroids needed as well as to shorten the course."This is one of the things that the CARRA (Childhood Arthritis and Rheumatism Research Alliance) network is studying right now," Reed said. In Europe, where methotrexate is not as readily available, physicians are more likely to use cyclosporine, but here, doctors use it sparingly to avoid possible kidney disease. "Also, it can cause a lot of hair growth, which of course the kids don't like."

IVIG

Intravenous immunoglobulin has become an accepted treatment, especially with chronic disease and cases where there's a lot of skin involvement, Reed said. "It's not usually offered at the start, but after the first or second month." JM patients usually get infusions every month.

Tacrolimus is a cousin to cyclosporine. "It can be useful, especially where there is lung disease. Reed also said she sometimes prescribes a tacrolimus ointment that's applied directly to the skin to treat skin disease.

Small studies have reported some usefulness for cellcept, but it hasn't shown itself to be comparable to methotrexate, and more cases of malignancy have been reported with its use.

Reed is keeping her eye on a drug called akinra, which has shown to be safe in adults with arthritis and is just beginning to be studied in children. "It may turn out to be a nice option," she said, "except that it has to be injected under the skin every day, which is uncomfortable, especially for children."

Reed is the primary international investigator for the pediatric portion of the rituximab study, part of the international RIM (rituximab in myositis) trials that have recently been completed. "I can't talk too much about this because it's still embargoed," she said. "I can tell you that patients do improve." She said rituximab is not a drug you would prescribe for serious, life-threatening disease. "It doesn't work fast, but it will be a useful option for children who need to get off steroids." Even though it is administered intravenously, it's only needed every 5-6 months, she said.

What's ahead?

Reed sees improvements ahead in stem cell therapy and bone marrow transplants. "As yet there have been some good and some bad results," she said. "We need a lot more information on this." In the few cases where these therapies have been used on children, researchers have had to suppress their immune systems.

Combination therapies will continue to be used, both to promote healing in different areas and to reduce the side effects of large doses of any one drug. International networks like CARRA enabled researchers to make tremendous strides in childhood cancer and cystic fibrosis treatment, dramatically lowering the death rate for both these diseases. By networking, pediatric rheumatologists establish reasonable protocols, share treatment ideas, and participate in joint trials. "This moves everything ahead much faster," she said.

What else is important?

- **Exercise**. "The thinking used to be just to put the kids to bed, but we know now that exercise not only makes children stronger, but it also promotes normal healing of the muscle fibers," Reed said. Unless the child is so weak that he or she may fall, let him or her be as active as they want. "Don't push them to run a mile," she said, "just encourage activities."

- **Diet**. Very important, she said, especially for children who are on prednisone. Lean protein, fruits and vegetables are needed to offset weight gain and promote healing. Reed recommends foods high in calcium and vitamin D, and berries as well as green tea, which are rich in anti-inflammatory agents.

- **Supplements**. Reed recommends fish oil supplements for heart-protective as well as anti-inflammatory

properties. Calcium and vitamin D are also important, especially for children who can't tolerate the sun.

- Sun protection. "Any of us with gray hair, who've been in this field a long time, believe that the sun is an influence on this disease," Reed said.

- Physical therapy. If there is stiffness, contractures, extreme weakness or tightness, your child may need some professional direction in an exercise program.

- Information. Get as much as you can, from online sources like TMA and CARRA, and by asking plenty of questions.

Disease course

There are three different scenarios, Reed said. They're all treated basically the same at first, since the physician doesn't always know which type your child will be. There are many different time frames but all have some similarities:

- Monophasic, where a child gets the disease, gets treatment, responds well in about two years, heals and remains disease-free in the future.

- Non-ulcerative chronic, where a child responds, but relapses and flares, improves and may flare again. Most cases are like this, Reed said, and physicians adjust treatment accordingly. Usually the child improves gradually and is in remission after 4 to 5 years.

- Ulcerative chronic, which physicians usually recognize from the start because of more breathing and swallowing difficulty, and treat accordingly with more aggressive therapy. Different drugs and combinations are tried, and the disease persists into adulthood.

■ ■ ■

The path that's right for you

Stephanie Yerhot, mother of a JM patient, spoke at the day-long conference at the Mayo Clinic. In between laughs and yoga poses, Yerhot had some good advice for remaining positive and healthy while coping with a devastating life change.

It's not always the same path, said Stephanie Yerhot. "If it helps you to know every bit of research that's been done on juvenile myositis, then read every study you can find." Yerhot found that this approach didn't help her. "I found I did better if I approached my daughter's illness in a more light-hearted way." Yerhot and daughter Ali went for ice cream, tried new things, laughed at life's indignities and found ways to get stronger together.

It wasn't always easy, Yerhot said. For some reason, Ali got into a blind panic about needle sticks. "I raised my children to be respectful and, most of the time, Ali was respectful." However, at the approach of a nurse with a needle, she screamed, hyperventilated, struggled and tried to run. "I literally had to get her in a body lock," Yerhot said. "I'd tell the nurse, 'you get one chance and we're out of here.'"

For parents, the worry is terrible, but Yerhot found the feeling of helplessness and guilt even worse. "She'd look at me like 'fix it,' 'fix it,' and I'd have to tell her I couldn't," she said. "We're moms, and that's what moms do."

She looked for ways for her daughter to feel powerful, so when Ali suggested martial arts, Yerhot -- after some initial shock -- decided to accompany her. "They tell you to figure out what you take away from each tragedy," she said: "I got a black belt."

Along the way, she also learned yoga, which she recommends to everyone, no matter what their approach to life's challenges may be. Don't be afraid of it, she said. "Yoga just means union. It's an ancient way of trying to get your body and mind together."

It's best not to have preconceptions about whether you can or can't learn yoga, she said. "Don't think you have to be a certain kind of person." For instance, Yerhot says she has a very busy mind. "So, I have a lot of energy, I'm creative, I have fun -- what's wrong with that?"

However, with practice, Yerhot was able to focus, to concentrate in a new way. "If you're doing a balance pose and you lose focus, you'll fall down," she said. It's not just with yoga, but with every movement that you have a chance to bring body and mind together. "For instance, if you're running and you just put on the earphones, you're missing a chance to focus on your movement and your breath. Whether yoga, running or just walking, if you're really at Target in your mind with your list, or at the grocery store, that movement is not helping you learn and grow."

In fact, she said, practicing yoga or any focused movement helps keep you younger. She used the analogy of an onion: "You peel the layers away to get at the center. Every time you do it, you take off another layer."

Finally, she said, she and Ali both learned something. “I couldn’t ‘fix it.’ But she knew we were with her. We were warriors, going down the same path.”

■ ■ ■

Peyton Hutchins: words shared

By Quineesa Smith

Peyton Hutchins is a juvenile myositis patient now in remission. The following is a summary of her talk in Minnesota.

Peyton Hutchins is like any 20-something. She lives in Washington, D.C., works for a non-profit organization that builds playgrounds for young children in disadvantaged neighborhoods, and has friends and family who are special to her. But, she doesn't take things the way they are now for granted. Peyton has juvenile dermatomyositis.

When she was diagnosed 14 years old, nobody knew what was wrong. Peyton already had some major medical challenges: She'd been born with a cleft lip and palate and had had several surgeries to fix the condition. For some time, she'd noticed some fatigue, but didn't think much of it. She chalked this up to "getting older," she says.

But during her third surgery, Peyton's doctor noticed a rash on her face, underneath her eyes and on her cheeks. Her parents, alert to any new medical issues, made a doctor's appointment and found out the weakness, fatigue and rash were due to JM. Luckily, Peyton lived in Baltimore and was able to go to Johns Hopkins Hospital for care.

Peyton has overcome many challenges in her young life, and she shared her story with the group of parents and children. She discussed being thought of as the "sick kid" at school and how she helped her friends and classmates understand her disease.

Peyton also told the story of her decision to go to school away from home and how her parents coped with her decision. Together, they came up with a medical plan in case anything went wrong. Peyton's school was close enough to a very good hospital (Vanderbilt) so that she could find doctors who knew how to treat her. At the same time, she had the independence of being away at college.

After completing her education, she moved back home, and now is in remission.

Peyton learned some key life lessons dealing with some of her challenges. She is grateful that her parents respected her choice, and also helped with some careful planning to keep her safe while she learned how to manage her disease. She encourages other families to do the same.

Some ideas from Peyton for young adults with JM and their families:

- Try community service. It's a way to stay active, while also doing something for someone else.
- It's going to be ok. This doesn't mean that everyone's outcome will be the same, but that acceptance is part of living with the disease.
- Learn to advocate for yourself. Remember that it's going to be up to you.
- Find the right doctor. Make sure your doctor is someone who works with you and who makes you feel comfortable.
- Don't let the disease define you. You are so much more than myositis!

Headed for college?

If you are looking ahead to college, there are some online resources that will help you, whether you need some accommodation for your illness or some shortcuts to conserve strength.

FAQs about Section 504 and Post Secondary Education
www.pacer.org/publications/adaqa/504.asp

Section 504 Subpart E: Postsecondary Education
www.ocean.edu/campus/student_services/drc/asd/mmedia/docs/minfo3.htm

"College Students and Disability Law" by Stephen Thomas.
Comprehensive article about Section 504, ADA, qualifying as a 'person with a disability', admission, accommodations, and dismissal. Includes guidelines for higher education practitioners; legal references.
www.ldonline.org/article/6082

Know Your Rights and Responsibilities
www.ed.gov/about/offices/list/ocr/transition.html

Answers on Disability Discrimination under Section 504 and Title II
www.ed.gov/about/offices/list/ocr/qa-disability.html

Policy Statement: Disability Harassment in Colleges (U.S. DOE)
www.wrightslaw.com/info/PolicyDisabilityharassment-2.pdf

Continued on page 8.

Am I different?

JM children solve problems that come with school

After a summer with family and close friends, it can be hard to be back in school. If you find that you hate the start of each new day, you may like some of the tips collected by JM kids, mothers, fathers, doctors and teachers from the book, “Myositis and You” and true stories from children and families. Here’s how some JM kids solved ordinary, everyday problems.

I look different from myself with a fat, red face.
If you think other kids are looking at you and laughing, maybe they just don’t know you are sick. Ask your parents to talk to your teacher and have a class discussion about JM. You may be able to put together a slide show or hand out information about JM. For help, email TMA@myositis.org; or go to www.myositis.org.

My clothes don’t fit me anymore.
Don’t throw them away! Most likely, they will fit you again soon. Ask your parents to help you get together a few “JM outfits” that you can wear until you’re back to your old size. Girls say they like smock tops and loose-fitting big shirts. Boys like sweats, if they’re allowed, and flannels over t-shirts.

I have to go the bathroom a lot more than the other kids.
This is something your parents can arrange with your teachers ahead of time so the teacher can help you do it without much notice. You should be able to go to the bathroom whenever you need to.

I am getting in trouble because I can’t get ready in time and am late a lot.
Again, your parents should talk to your teachers so you don’t get in trouble for being late. Try doing everything you can to get ready the night before. Alex said he showers at night, puts on clean clothes that don’t wrinkle, and is ready in five minutes even though he feels weak and stiff in the morning! His mother keeps cereal bars and juice packs in the car and they make a game of getting to school on time. She had him do stretching exercises to music during their 20-minute drive. See what crazy ideas you can think of.

It’s hard for me to carry my books.
Ask to keep a set of the heaviest books at home. That way, you can just bring your homework assignments back and forth.

After School Snacks!

Banana Nut Pudding
Need: 3 bananas, 1 1/2 cups applesauce, 3 tablespoons peanut butter, 2 teaspoons lemon juice, 6 paper cups, blender.

Mix all the ingredients in a blender. Spoon into cups. Place a few bananas on top. Refrigerate before serving.

Sun Baked S'mores
Need: graham crackers, foil pie plate, aluminum foil, chocolate chips, miniature marshmallows.

Place graham crackers flat in a foil pie plate, sprinkle with chocolate chips and miniature marshmallows. Lay another graham cracker on top. Cover the pie plate with aluminum foil. Place the covered pie plates outside in a sunny hot place. Check after about 10 minutes. They are done when the sun has melted the chocolate and marshmallows.

Word jumble

1. lagf 2. reachte 3. lappe
4. sokob 5. ssrcoomla
6. cllsooh 7. neplic
8. mweokrho 9. hlkac
10. erapp 11. snpe
12. llatcucrao

1. Flag 2. Teacher 3. Apple 4. Books
5. Classroom 6. School Bus 7. Pencil
8. Homework 9. Chalk 10. Paper
11. Pens 12. Calculator

College Resources

Continued from page 6.

College: Continuing and Higher Education for Students with Disabilities - Wrightslaw
www.wrightslaw.com/info/college.index.htm

Financial Aid Guides and Resources – Wrightslaw
www.wrightslaw.com/info/fin.aid.index.htm

Steps to College
www.nacacnet.org/MemberPortal/News/StepsNewsletter/

Study Strategies
www.d.umn.edu/student/loon/acad/strat/

Study Skills Guide
http://www.csbsju.edu/academicadvising/helplist.htm

Ten Traps of Studying
http://campushealth.unc.edu/index.php?option=com_content&task=view&id=470&Itemid=65

Sample 504 Plan: Accommodations in the Classroom and for Testing
www.ocean.edu/campus/student_services/drc/ADA%20_form.pdf

Cornell Note Taking System
www.clt.cornell.edu/campus/learn/LSC_Resources/cornellsystem.pdf

Self-Advocacy for College Students
www.ldonline.org/article/6142

Planning for Successful Transition for All Students
www.heath.gwu.edu/node/338

The Myositis Association

1737 King Street, Suite 600
Alexandria, VA 22314