Answered patients-questions for the Q&A Session at the GCOM 2019

Correlation between Myositis and other diseases/symptoms:

1) Copper storage disease - related to myositis? - treatment options?

   Answer:

   Myopathy possible, myositis not really proven. Therapy with copper chelators and uptake inhibitors can work, but no healing

2) Before my diagnosis of sIBM, I was seriously ill with exogenous allergic alveolitis (EAA), which could be stopped with cortisone and is currently resting, but would become active again when aroused. Are compounds of these two diseases known?

   Answer:

   Not known

3) Is there any correlation between myositis and sleep apnea?

   Answers:

   I. Yes, possible
   II. But rare.

4) Is there any connection between AIHA and polymyositis?

   Answers:

   I. Only few case reports
   II. Rare

   a) Can tooth decay caused AIHA and/or polymyositis?

      Answer:

      No

   b) What is live expectancy of polymyositis and AIHA like me?

      Answer:

      No exact numbers but if treated well, if all works well no reduction possible

   c) Beside AIHA and polymyositis, I've diagnosed with COPD, asthma, hypertensive heart disease, and GERD. Is there a correlation among these diseases?

      Answer:

      Not proven with large cohorts, but possible

5) With sIBM. Is it normal for autoimmune diseases to occur prior to diagnosis of the disease.

   Answer:

   Yes

   Is it typical for additional autoimmune diseases to occur after sIBM diagnosis.

   Answer:

   Not really, but possible

   I was just diagnosed with Lupus after 2012 confirmation of sIBM.

   Answer:

   That’s unusual, both diseases should be reconfirmed to show/exclude overlaps

6) I also have an HIV positive diagnosis and IBM. Although I'm extremely healthy with t cell counts higher than those without the disease, I still feel as if somehow the HIV caused my IBM. Is there

any research around IBM as it relates to HIV and could the HIV meds be causing the muscle weakness?

Answers:

I. There are only few case reports showing this combination, so it´s unlikely, but possible
II. Some (older) meds can cause mitochondrial damage HIV and sIBM is a rather rare but well established coincidence viral diseases can cause sIBM (Hepatitis B and C as well)

7) Is restrictive lung disease seen as part of polymyositis or another type of myositis?

   Answers:

   I. No
   II. Different subtypes of interstitial lung diseases can be associated with anti-Synthetases syndrome and this makes restriction!

8) Please comment on the link between IBM and LGL Leukemia. I have both and have read that there are others like me.

   Answers:

   I. Very rare combination, both linked to pathogenic cytotoxic CD8+ T cells
   II. LGL lymphocytes are associated with sIBM in variable quantitaty (sometimes many sometimes very few or none BUT this does not mean that all sIBM patients have LGL leukemia, conversely not all LGL leukemia patients will get sIBM but some (very few!) have been described and at younger age than 'normally' possible

9) Have any of the experts heard of a patient with dermatomyositis (with ILD) who is also being treated for a thoracic aortic aneurysm, and POTs? Do they have any thoughts, or is there any published literature or studies on whether these conditions are somehow related, and/or treatment options?

   Answer:

   No, this combination is by chance

10) 26 years old diagnosed one year ago with polymyositis in addition to interstitial lung disease (ILD). I'm from the United Arab Emirates. Due to lack of knowledge regarding such disease around here, I'm the only one around who has it.

    Answer:

    This is probably Antisynthetase syndrome any autoAB?

    a) What do I need to expect in future?

       Answer:

       Intensive check-up, try to ameliorate the manifestations with adequate medication asap

    b) Will I keep feeling this continuous pain?

       Answer:

       With therapy, it should become better

    c) Will my joints be affected?

       Answer:

       That could happen in an overlap situation

    d) Will I have the chance to live like any other lady without difficulties, like having kids for instance?

       Answer:

       Yes, but at first a remission has to be achieved

11) My doctor says he is 80% sure I have IBM. I was simultaneously diagnosed with breast cancer, which has since been treated.
    a) Is there any treatment, experimental or not that I can try for IBM?

       Answer:

       There are clinical trials running but most likely, tumors will be an exclusion

    b) Also, will any of these treatments interact with my Tamoxifen?

       Answer:

       Not known

    c) Are there any clinical trials available for women?

       Answers:

       No

       There are clinical trials, however inclusion is not dependent on gender

12) Has there been any evidence of myositis affecting either heart or bowel muscles?

    Answer:

    Yes

IBM: Research and Treatment

1) In 2018, researchers achieved success in Alzheimer's disease: the antibody BAN2401 slows down the course of the disease. (BAN2401 monoclonal antibody, which removes amyloid beta-protofibrils from brain glial cells, preventing neuronal death in a Phase 2 trial, reduced brain plaque formation and slowed dementia progression.)

   Can this research step (antibody) be applied to the clinical picture sIBM "sporadic inclusion body myositis" or further researched? (Quote of a Prof. in Germany: "In the muscle messenger substances (cytokines) such as interleukin-1β are released." Parallel to the inflammation, the muscle recovers due to various proteins that are deposited in the muscle fibers It also plays a role in diseases of the brain, most commonly deposits of the protein ß-amyloid, which also occurs in Alzheimer's disease. ")

   Answers:

   I. Not known, but off label an interesting idea
   II. For Alzheimer’s disease there is so far no convincing data for the efficacy of amyloid-targeted therapies, maybe due to late induction of therapy. For sIBM effects are not known, but might mechanistically justified and subject of further studies
   III. ß-amyloid is is a completely different mechanism in Alzheimers disease. There is no or very scarce **beta** amyloid in sIBM! The fibrils are beta pleated tau

2) With possible good results from the arimoclomol trial at the moment, what help and at what progression of ibm will it prove beneficial to later stage ibmers. Will it stop the progression or even help to regain what I’ve already lost with strength and mobility?

   Answer:

   That’s what the trial needs to prove

3) Does IBM affect my heart, because heart is also a muscle.

   Answers:

   I. Yes, possible, but not proven
   II. In Literature, no affection of heart has been described, but I have 5 IBM patients, of whom all of elevated troponin t (a heart-specific muscle enzyme), but further diagnostics are without pathologic (MRI, echokardiographia). However, since IBM patients are older (>60 years) and the risk for heart problems is already increased. From my point of view, there is no need to concern for the heart of IBM patients. There is only a laboratory hint, but no clinical evidence.

4) Is there any treatment or medication for IBM disease?

   Answers:

   I. Yes, please see the IBM trials at clinicaltrials.gov
   II. (https://clinicaltrials.gov/ct2/results?cond=IBM&term=&cntry=&state=&city=&dist=)
   III. Sometimes, intravenous immunoglobulins slower disease course. Until now, physiotherapy is the most helpful therapy. Another medical treatment that has fulfilled criteria of effectiveness in school medicine are not available.

5) New cancer treatments now include targeting specific genes and cells (immunotherapy) with some amazing results (think of past president, Jimmy Carter's brain cancer). Is that research applicable to addressing s-IBM?

   Answers:

   I. Potentially yes, but unlikely at present
   II. Potentially yes, but unlikely at present, targeted therapies are a major goal in myositis research
   III. Since the cause and mechanisms of the disease are to less known and studied, we cannot answer whether immunotherapy will bring relief. Immunosuppression with Prednisolone and more does not help

6) Does anyone have any experience with the influence of procaine infusions on sIBM? I was right after the biopsy of a muscle on the left Lower leg with this preparation an already dramatically to name increasing strength and especially mobility of the leg muscles to notice, however, only about a day. I was able to reproduce the effect later. A Hamburg doctor injected me with 5 mL procaine 1%, i.V. The effect was repeated.

   Answers:

   I. That has been discussed years ago but never been followed
   II. I have no experience.

DM Research and Treatment

1) I was diagnosed with Dermatomyositis with a muscle biopsy several years ago. I have been taking 5 to 7 and half mg. of Prednisone.
   a. Can you tell me if there are certain things that trigger an episode?

      Answer:

      Yes, any infection can trigger

b. If I knew, could I avoid the swelling and rash? I have suspected heat or sunshine. Perhaps antibiotics also. (USA)

   Answers:

   I. Immunosuppressants may be better than steroids
   II. ; heat or sunshine might worsen the symptoms in some cases
   III. Please avoid sunm, as it triggers the rash. When the rash is there topical use of so called calcinnurin inhibitors (e.g. pimecrolimus ointment) is helpful or oral intake of quensyl

2) I have been diagnosed with dermatomyositis. Since a couple of years I am suffering from really painful calcinosis. Could you tell me if there is any news on insights or treatment options of calcinosis?

   Answers:

   I. No, unfortunately not
   II. ; currently sufficient immunosuppressive therapy is the best option
   III. ,but does not act on calcinosis in many cases as well
   IV. It depends also on size for small (1-2mm) deposits there are reports on successful use of a prescription containing sodium thisosulfate, which corresponds to our experience

3) Dermatomyositis diagnosis made Oct 2017 and treated with Rituxan and Physical Therapy successfully. Based on my case history with eventual lung cancer being diagnosed, my question would be should cancer screening be routinely part of the protocol for treatment?

   Answers:

   I. Yes
   II. Especially when certain antibodies are found in the blood (NXP-2 and TIF-1)

PM Research and Treatment

1) Is there any new treatments for polymyositis both newly Dx and resistive to treatment cases?

   Answers:

   I. Not licensed, but **all** immunosuppressants used for rheumatic diseases are being examined
   II. Not licensed, but **many** immunosuppressants used for rheumatic diseases are being examined

2) Is there any new list of symptoms to diagnose polymyositis?

   Answers:

   I. No
   II. A diagnosis of PM should be avoided we can be more precise

3) Are there new diagnostic criteria for Polymyositis with subtypes?

   Answer:

   No

4) Are there new specific treatments for Polymyositis?

   Answer:

   See above

5) Are there natural treatment methods (alternative treatments)? How close are they to a cure?

   Answers:

   I. Not available
   II. , so far no natural treatment has proven any substantial effect on autoimmune diseases

6) I understand exercise is very good for polymyositis patients. I also can feel the benefits of exercise sometimes. I am in pain most of the time especially around my shoulders and neck area. Should I continue to exercise, or I am making more damage to the muscles and joints in this area? OR, is there hope that the muscles/joints will improve with exercise hence lead to reduction or no pain in the future?

   Answers:

   I. If pain is persisting, activity should be checked and therapy adapted
   II. The recommended intensity and the form of exercise depend on the activity of PM in your case. If there is more disease inflammation activity and you need a higher dose of corticoids you have to be more careful und exercise with less effort should be done, using an aerobic exercise. If the course of the disease is more stable and you need a low dose of corticoids, you can be more active, perhaps even with a resistance training.

7) My diagnosis is Polymyositis. I have been taking Prednison for over 17 years now. Can you tell me if there is a specific treatment or medication for PM yet?

   Answers:

   I. Yes, immunosuppressants such as MTX and others
   II. Yes, there are immunosuppressants such as MTX and others, however specificity is rather low

NM Research and Treatment

1) How is the remission of HMA Co reductase autoantigen necrotizing autoimmune myopathy defined? Where do my CK levels and other inflammatory markers need to be at to say I am in remission?

   Answers:

   I. It`s thought to be autoantibody mediated, remission is normal CK levels and no symptoms
   II. Remission should be measured clinically. Biomarkers can b helpful such as CK or auto AB titers. In anti-HMGCR-myositis CK levels stay elevated often and titers can correlate with clinical amelioration

2) With necrotizing myopathy, how many grams of protein, carbs and fats should I shoot for daily?

   Answer:

   Not known, but should not make any difference, as the process is autoimmune

   a. What is sane and realistic BMI for me? I am 5' 1 ....137 pounds, 59 years old.
   b. What about supplements? Lately my CK levels have great. I want to know if 5 grams of creatine monohydrate a day is a good idea or not?
   c. What about bone building formulas for osteopenia? Think I am right on the cut off line for osteoporosis. How do I address THAT?

      Answers:

      I. Treatment according toosteoporosis guidelines
      II. ; for beginning osteoporosis vitamin D and calcium supplements might be an option

3) What is a realistic bone building program for someone with osteoporosis and NM?
   Answer:
   Standard physical therapy

Genetics:

1) I was wondering if there has been any research into possible genetic predispositions to myositis? I know that there is some talk about certain genetic mutations can lead to inflammation, which may cause autoimmunity problems. These then can manifest in various autoimmune diseases such as Lupus, MS, psoriasis and, perhaps, myositis diseases. Could there be any possibility of this?
   Answers:
   I. Yes, but different in most of the individuals
   II. ; in autoimmune disorders there are always genetic and environmental factors contributing to the pathogenesis, genes might be risk factors but are not sufficient to initiate disease alone

physical therapy/ergotherapy

1) In the last year the muscle loss has increased strongly and the stiffening of the fingers begins, so that problems occur during many activities. I go to occupational therapy twice a week, physiotherapy twice a week and speech therapy once a week? Since the muscle reduction continues to progress, especially in arms and legs, my question is:
   a. Are there special therapies that can be applied?
      Answers:
      I. No, but the immunosuppressive therapy needs to be adapted
      II. ; physiotherapy should be adapted to your symptoms as well, however additional physical training at home might be even more important
      III. The recommended intensity and the form of exercise depend on the activity of myositis in your case. If there is more disease inflammation activity and you need a higher dose of corticoids you have to be more careful und exercise with less effort, using a mild to moderate aerobic exercise. If the course of the disease is more stable and you need a low dose of corticoids, you can be more active, perhaps with a resistance training. And the intensity of the resistance training as well depends on the body conditions. As you describe muscle loss especially in arms and legs and stiffening of fingers there seems to be a high disease activity. So passive movements of your hand and finger joints should be done with your therapists and on your own in an everyday exercise program, using the whole extent of motion of the joints. Active movements of the whole body muscles and joint should be done in your everyday exercise with low effort. The intensity and time of exercise should be discussed individually with your physician and your therapists.

2) IBM is always very rare: Are there any new findings for a possible therapy, physiotherapy and speech therapy and lots of exercise... for example infusion with immunoglobulins?

   Answers:

   I. IVIGs have not proven efficiency in IBM; but check clinicaltrials.gov for trials to participate
   II. (https://clinicaltrials.gov/ct2/results?cond=IBM&term=&cntry=&state=&city=&dist=)
   III. H. Alexanderson summarizes in a recent review (Exercise in Myositis; Curr Treat Options in Rheum (2018) 4:289-298) according to sIBM: "As of today, we do not know what exercise regimen is most effective in IBM; however, evidence supports safety of individually adapted exercise and that regular exercise can maintain function while inactivity leads to reduced muscle function". She further stated, that blood-restricted resistance training (BRF) could be an effective exercise form for sIBM patients. Larger randomized controlled studies are needed to decide whether this kind of exercise should be used regularly in sIBM patients. Further studies have to be performed as well to clear whether intensive exercise should be combined with medications, for example Bimagrumab.

3) What exercises do you recommend for IBM?

   Answers:

   I. Standard physiotherapy
   II. Depending on literature and own experience I recommend for IBM patients a combination of aerobic exercise and resistance training individually adapted. These exercise forms are very common and available. Overstrain should be avoided.

4) Can you gain back muscle lost thorough physical therapy?

   Answers:

   I. Yes
   II. Getting back muscles lost cannot be expected. The aim must be to improve strength and functional capacity, stabilization of preserved muscles and to slow down process of muscle wasting and loss of strength.

General questions:

1) I've been diagnosed with autoimmune hemolytic anemia since 2016. My hematologist gave me Sandimmune Neoral, methylprednisolone, antacid, and braxidin. Recently, my doctor suggested he check my blood with ANA Test. The result is... I am positive for polymyositis.
   My questions are:
   a. What is the difference between "myositis" and polymyositis?

      Answers:

      I. Poly just means "more than one muscle affected"
      II. ; there is no blood test saying you are positive for polymyositis, this is a diagnosis made by biopsy, I would recommend to consult a myositis center
   b. I have been told I will need prednisone for one year. Does Myositis go away in one year?

      Answers:

      I. No
      II. There are acute and chronic forms of myositis, therefore there is no clear answer to your question

2) If you had IBM, how would you treat yourself?
Answer:
Try to get into a clinical trial, see above

3) I was diagnosed with IBM about 2 years ago. I have noticed increased muscle burn when exercising which I do regularly. If I push through the muscle pain, am I making my condition worse?
Answer:
No, but don't push it to the limit

4) Are there any type of myositis that presents with a drop head?
Answers:
   I. No, but neck muscles can be affected
   II. No, **usually not,** but neck muscles can be affected
   III. iR Myositis is axial all the others can develop dro head during disease course- but rarely. Drop head is typical for SLONM which is not myositis

Standards on myositis:

1) When a patient is pursuing a diagnosis of myositis, is there a recommended list of blood tests including antibody tests to be undertaken?
Answers:
   I. Yes
   II. (We normally do CRP, CK, TSH, Na^+, K^+, Ca^{2+}, ANA, anti-dsDNA (with typical symptoms for SLE), (pANCA, cANCA), myositis-associated antibodies and myositis-specific antibodies

hIBM:

1) Is there a difference between treatment options being considered for those with genetic IBM vs those with sporadic IBM? If so, why are the options different?
Answers:
   I. At present, no specific options
   II. For hIBM can try sialic acid

2) What is the newest information on Familial Inclusion Body Myositis? I have IBM, my mother had IBM and I had one sister diagnosed with Polymyositis. I also have an older brother who is not affected at all. Has any connection been found?
Answer:
There should be a genetic but family-specific link

3) Why is there not much interest with patients who are suffering with HIBM. We are of a more rare condition yet need help as well. I feel we are the forgotten ones.
Answers:
   I. Its not forgotten, just too many similar problems for the few experts
   II. My suggestion is to go and see an expert for hIBM depending on your country we can help and give names

other medications for myositis possible?

1) Can the Myositis Doctor community find better solutions than high dose, long term Prednisone?
   Prednisone has been around for over 100 years.
   Seems like we should be able to find a better treatment plan, especially for those of us who have Osteoporosis or other steroid sensitive co-morbidities)
   Answer:
   Yes, there are multiple immunosuppressive options and IVIGs