

Dear colleagues,

the Wiskott-Aldrich syndrome (WAS) is a complex disorder with a wide range of disease severity and unique hematological and immunological manifestations. Based on this complexity we have several different therapies to offer to WAS and X-linked thrombocytopenia (XLT) patients, some purely symptomatic, some potentially curative, some associated with serious risks. In many instances more than one of these therapeutic options seem appropriate for any given patient.

A prospective, randomized study comparing the pros and cons of these therapeutic options in WAS/XLT will never be conducted for a multitude of reasons. But in order to counsel patients properly we should know the consequences of these therapies and how they affect patients' quality of life.

With the generous support of the Israeli WAS association and the US WAS foundation we therefore ask for your help in conducting an international survey to assess, in a retrospective manner, the frequency of disease and therapy related complications with respect to the specific treatment received. It is our goal to collect anonymized data from as many WAS/XLT patients as possible in order to be able to draw relevant conclusions.

If you would like to help us achieve this goal, we ask you to complete and return the attached short case report form - one for each of your patients - to us by fax, e-mail or postal mail. For your convenience you may also use the fillable PDF which can be opened with any version of the free Adobe Acrobat Reader ([download Adobe Reader](#)). You can save each of your patients' data in a different file and edit it as often as needed. (See attached instructions for help).

A few notes:

- we are interested in all WAS/XLT patients, irrespective of whether they have undergone splenectomy, stem cell transplantation, gene therapy, multiple or none of these procedures.
- the survey was specifically designed not to interfere with other current on-going studies by the WAS family organizations, the PIDTC and others.
- data are collected anonymously and retrospectively by chart review, so in many institutions the study may be IRB exempt or expedited.
- we highlighted in yellow those areas of the survey which we consider to be essential information. Please pay special attention to supply (approximate) dates, otherwise it will be impossible to analyze the data.
- feel free to add additional information in the "comments" box on page 3 section or anywhere on the form if you prefer.
- authorship on a manuscript originating from this study shall be based on number of patients included by each center.

Thank you very much for your help, looking forward to a fruitful collaboration. Please feel free to contact us with any questions about the survey at [was-survey@med.lmu.de](mailto:was-survey@med.lmu.de).



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