UPDATE

JANUARY 2017

A-T Clinical Center at Johns Hopkins Hospital

In this issue:

- *Immunizations*
- *Dexamethasone Trial*
- *Follow up Visits*

Contact Information

The Johns Hopkins Hospital
600 N. Wolfe Street CMSC 1102
Baltimore, MD 21287-3923
Phone: 410-614-1922
Fax: 410-955-0229

DIRECTOR/IMMUNOLOGY
Howard M. Lederman, M.D., Ph.D.

RESEARCH NURSE/ COORDINATOR
Jenny Wright, R.N.

DATA MANAGER
Renee Allard

NEUROLOGY
Thomas O. Crawford, M.D.

PULMONARY
Sharon A. McGrath-Morrow, M.D.

SPEECH-LANGUAGE PATHOLOGY
Maureen Lefton-Greif, Ph.D.

NUTRITION
Caitlin Krekel, R.D, L.D.N.

News about immunizations for people with A-T

- **There are new data linking vaccine-strain rubella to chronic granulomatous skin lesions (Perelygina L, et al. 2016. J Allergy Clin Immunol 138:1436-1439) in individuals with A-T. Therefore, we recommend that individuals with A-T do NOT get the measles/mumps/rubella (MMR) vaccine. (Please note that there is only minimal risk for developing granulomas if you have already received the MMR vaccine.) Patients of our clinic can contact us to receive an exemption letter for the school system as necessary. If an unvaccinated person with A-T is exposed to someone who has measles or mumps, we recommend that he/she receive gamma globulin to prevent infection. This is not necessary for people already receiving gamma globulin therapy on a regular basis.**
- Individuals with A-T and all household contacts should be immunized with the influenza vaccine (killed or injected) this and every year!
- Individuals with A-T should be immunized with the pneumococcal conjugate vaccine (Prevnar-13®) at 5-year intervals in an effort to maintain high levels of anti-pneumococcal antibodies. They should also be given at least one dose of the 23-valent pneumococcal polysaccharide vaccine (Pneumovax®) after the age of 2 years.
- Varicella vaccines are safe and should be administered at the regular schedule.
- Individuals who are getting gamma globulin therapy (IVIG or SCIG) do not need any vaccines except for annual influenza (killed or injected vaccine).

The A-T Clinical Center will be sending out updates to help us keep in touch with individuals with A-T around the globe. We welcome your questions, comments and suggestions. Please send them to Jenny Wright, RN (jenny.wright@jhmi.edu), Nurse Coordinator.

To be added to the distribution list for future updates, please email your request to:

a-tclinic@lists.johnshopkins.edu

UPDATE

JANUARY 2017

A-T Clinical Center at Johns Hopkins Hospital

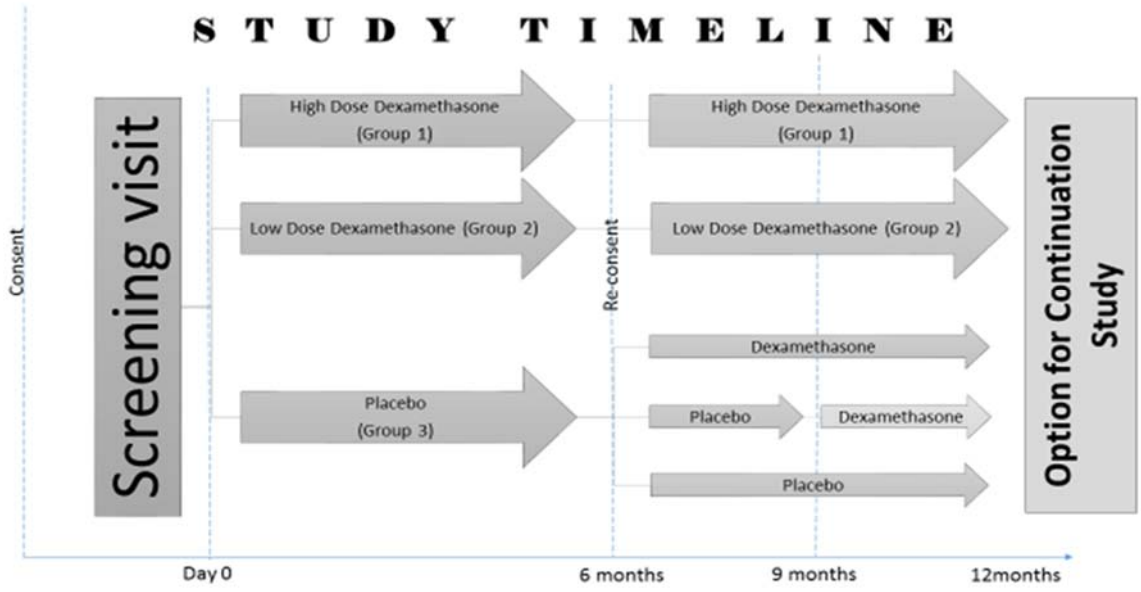
EryDel trial of steroid (dexamethasone) loaded into red blood cells

In 2017, EryDel, a company from Italy, will open a drug trial to determine if dexamethasone is safe and effective in treating the neurological symptoms that occur in A-T.

To be eligible for the study, a participant must:

be diagnosed with A-T,
be at least 6 years old,
weigh at least 33 pounds (15 kg),
be able to walk approximately 30 feet independently or with periodic use of support,
not be enrolled in another drug trial for the duration of this study.

The study consists of 2 parts, each lasting 6 months. In the first part of the study, participants will be randomly assigned to receive either the dexamethasone or a placebo (something that looks like the study drug, but has no activity). Participation in this trial will require monthly day-long visits for diagnostic tests, placement of an IV for withdrawal of 2.5 tablespoons (50 mL) of blood that will be processed in a laboratory to load study drug into the red blood cells, and re-infusion of the dexamethasone or placebo-treated red blood cells later the same day. Over the course of the study, participants will be required to visit the study site at least 16 times. All but two of the visits will require a full day at the study site.



To be added to the distribution list for future updates, please email your request to:

a-tclinic@lists.johnshopkins.edu

UPDATE

JANUARY 2017

A-T Clinical Center at Johns Hopkins Hospital

(cont.) EryDel trial of steroid (dexamethasone) loaded into red blood cells

Participants will be divided into three groups. Group 1 will receive a 14-22 mg dose of dexamethasone. Group 2 will receive a 5-10 mg dose of dexamethasone. Group 3 will receive placebo. Therefore, each participant will have a 2 in 3 chance of receiving study drug and a 1 in 3 chance of receiving the placebo. Neither the participants nor the doctors will know which study drug has been assigned to them.

After 6 months in the study, all participants who complete the designated procedures and tests will be eligible to continue in an additional 6-month study to collect information on the long-term safety and benefits of the study drug. Participants originally assigned to a dexamethasone group will continue on the same drug dose, while participants in the placebo group will be progressively shifted to receive dexamethasone.

After completion of the one-year study, all participants will have the option to continue to receive dexamethasone as part of a separate, on-going study.

Johns Hopkins Hospital (Baltimore, MD) will be one of four sites in the United States that will be participating in the trial. *Details can be found at attest-trial.com and at clinicaltrials.gov with the identifier:* **NCT02770807.**

~ Continuing care for individuals with A-T ~

We have a new understanding about the diagnosis and management of lung disease.

We understand the critical importance of attaining and maintaining normal weight for age. Being underweight adversely impacts neurologic function. It can also affect overall health and well-being.

We have updated recommendations for routine health screenings for adults with A-T.

If it has been more than 5 years since your last visit to the A-T Clinical Center, please contact us to set up a follow-up appointment.

To be added to the distribution list for future updates, please email your request to:

a-tclinic@lists.johnshopkins.edu