

AORTIC VALVE OPERATIVE OUTCOMES IN MARFAN PATIENTS
Multi-Center Study

Background

Two different surgical operations are used to restore the function of the impaired aortic valve: aortic valve replacement (AVR) and aortic valve sparing (AVS). AVR uses mechanical or biological valves to replace patient's natural aortic valve. AVS repairs and retains patient's natural valve. As your tests indicate, you may need to undergo one of these operations. It is not clear which of the two operations is better for patients with Marfan syndrome because this has not been researched in full before. Current study will compare results of AVR and AVS operations and use this information to help the doctors in choice of operation. This is the reason you are asked to get enrolled into the study.

This research study is sponsored by National Marfan Foundation, Baylor College of Medicine, other participating institutions.

Purpose

The purpose of the study is to observe and compare operative outcomes of the AVR and AVS surgeries in different categories of patients with Marfan syndrome and determine which factors have impact on surgery outcome.

Procedures

A total of 250 subjects at 15 institutions will be asked to participate in this study. You will be one of approximately 17 subjects to be asked to participate at this location.

The research will be conducted at the following location(s): Baylor College of Medicine.

There will be no changes to your treatment if you get enrolled in this study. Qualified study personnel will observe the course and results of your treatment and add few additional procedures that will not interfere with your treatment (see section Additional Procedures below).

Study organization

Leading surgical centers specializing in aortic root surgery in USA, Canada and Europe will join their efforts to complete this research. Study Coordinating Center will be located at Baylor College of Medicine, Houston, TX. The expected duration of the study is 3 years.

Description of treatment**Standard treatment procedures**

You will undergo an aortic valve operation as assigned by your doctor. It will restore proper performance of your aortic valve and fix other heart and aortic problems if needed. You will have a number of tests for this operation. Blood work, clinical evaluation of your heart and blood vessels, and echocardiogram will be performed before the operation, before your discharge from the hospital and during the follow-up period. Echocardiogram will be taken during the operation as well. You will have computer tomography of your heart and aorta performed before the operation. Additional tests may be requested by your doctor based on your personal medical needs.

After the operation your doctor will regularly check your heart and aorta. You will be requested to come for the follow-up visit in six months after the operation and yearly thereafter. Follow-up

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visits are very important because they allow your doctor to monitor your progress and prevent complications. All described procedures including follow-up visits are the accepted standard of care and will not require any additional effort from you. You may come for follow-up visits to your operating surgeon or to the other doctor. In this case the study personnel will request the follow up information from your doctor.

Additional procedures

The follow-up period for this study will last 36 months, or till the end of the study, whichever is first.

Your participation in the study will include the following additional procedures:

1. Signing the informed consent form, that you are now reading

This form explains the study goals and procedures, your rights and responsibilities and is a prerequisite of any research effort involving human subjects.

2. Confirmation of Marfan syndrome diagnosis.

2a. If this diagnosis has already been verified by other physician / medical institution, the study site will require you to provide medical documentation of the diagnosis, including result of genetic testing if done, and family history of disease.

2b. If you do not have the diagnosis confirmed before or do not have appropriate medical documentation, study personnel will refer you to the study site specialists. Diagnostic procedures will include complete physical examination; collecting detailed medical and family history; eye examination with a slit lamp after fully diluting pupil performed by the ophthalmologist; and echocardiogram looking for the involvement of aorta and major arteries. If results of any of these exams are already available from other medical institutions, they will be accepted for this study and not duplicated at the study center. If the diagnosis of Marfan syndrome is confirmed, you will be enrolled into the study. If diagnosis is not confirmed you will not be enrolled into the study.

3. Completing questionnaires.

During your preoperative visit(s) you will be offered to complete two questionnaires. The first one is health-related quality of life SF-36v2 questionnaire that will take you 5 to 10 minutes to complete. The second one will be short demographic questionnaire that will take you approximately same time to complete. You will fill out quality of life questionnaire second time twelve months after the operation.

4. Collection of study samples.

During the time of the operation tissue and blood samples will be collected. You will not have any discomforts related to collection of samples since you will be under anesthesia at that time. Blood sample will include about 4 teaspoons of blood for adults and children 2 years and older. For children less than 2 years of age the amount of blood will be smaller and in proportion to child's weight. Tissue sample will consists of small part of removed aortic wall (1 to 2 square centimeters) that is left over tissue. Genetic testing and / or immunohistochemical analyses will not be performed in this study. The samples and clinical information may be shared with other investigators working to understand aortic disease in patients with Marfan syndrome. These scientists will not, however, have access to your personal identification information (e.g. name, address, social security number, etc.). You will never be personally identified in any report of study findings, and the study data will not be entered in your medical record.

After study termination samples may be stored for a prolonged period of time until they are used

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completely for the research purposes. If you decide to have your samples removed you will need to contact the study staff and request sample removal in writing. In this case samples will be destroyed. You will receive a notification of sample destruction.

Please confirm:

1) You will be able to come for follow-up visits if your health condition permits:

Yes No Initials

2) You allow requesting follow-up medical information from your doctor if you will have follow-up visit outside the operating facility:

Yes No Initials

3) You agree for collecting your blood and aortic wall study samples as described above:

Yes No Initials

You can see and get a copy of your research related health information. Your research doctor may be able to provide you with part of your information while the study is in-progress and the rest of your information at the end of the study.

Potential Risks and Discomforts

No additional risks / discomforts from research procedures are expected. Protection of possible breach of confidentiality is described in section Subject's Rights.

Potential Benefits

The benefits of participating in this study may be: knowledge-oriented. Results of the study will allow clarifying what operation will provide best results for different subgroups of Marfan patients. Quality of life information will demonstrate future patients that may include your relatives what to expect in the aftermath of different aortic valve operations. However, you may receive no benefit from participating.

Alternatives

You may choose to not participate in this study.

Subject Costs and Payments

There are no costs to you subsequent to your participation in this research study. You will not be paid to participate in this research study.

Subject's Rights

Your signature on this consent form means that you have received the information about this study and that you agree to be a part of the study.

You will be given a copy of this signed form to keep. You are not giving up any of your rights by signing this form. Even after you have signed this form, you may change your mind at any time. Please contact the study staff if you decide to stop taking part in this study.

The investigator or sponsor may decide to stop you from taking part in this study at any time.

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You could be removed from the study for reasons related only to you (for example, if you move to another city, if you do not take your study medication, or if you have a serious reaction to your study medication) or because the entire study is stopped. The sponsor may stop the study at any time.

There may be unknown risks/discomforts involved. Study staff will update you in a timely way on any new information that may affect your health, welfare, or decision to stay in this study.

If you are injured because of this study, you will receive medical care that you or your insurance will have to pay for just like any other medical care. You will not be paid for the injury.

Your Health Information

We may be collecting health information that could be linked to you (protected health information). This protected health information might have your name, address, social security number or something else that identifies you attached to it. Federal law wants us to get your permission to use your protected health information for this study. Your signature on this form means that you give us permission to use your protected health information for this research study.

If you decide to take part in the study, your protected health information will not be given out except as allowed by law or as described in this form. Everyone working with your protected health information will work to keep this information private. The results of the data from the study may be published. However, you will not be identified by name.

People who give medical care and ensure quality from the institutions where the research is being done, the sponsor(s) listed in the sections above, representatives of the sponsor, and regulatory agencies such as the U.S. Department of Health and Human Services will be allowed to look at sections of your medical and research records related to this study. Because of the need for the investigator and study staff to release information to these parties, complete privacy cannot be guaranteed.

The people listed above will be able to access your information for as long as they need to, even after the study is completed.

If you decide to stop taking part in the study or if you are removed from the study, you may decide that you no longer allow protected health information that identifies you to be used in this research study. Contact the study staff to tell them of this decision, and they will give you an address so that you can inform the investigator in writing. The investigator will honor your decision unless not being able to use your identifiable health information would affect the safety or quality of the research study.

The investigator, [Investigator's name] , and/or someone he/she appoints in his/her place will try to answer all of your questions. If you have questions or concerns at any time, or if you need to

CONSENT FORM

HIPAA compliant

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report an injury related to the research, you may speak with a member of the study staff: [Investigator’s name], M.D. at [Investigator’s phone number] during the day and [Contact’s name], at [Contact’s phone number] after hours.

Members of the Institutional Review Board for [Name of the surgical center] (IRB) can also answer your questions and concerns about your rights as a research subject. The IRB office number is [].

If your child is the one asked to take part in this study you are signing to give your permission. Each child may agree to take part in a study at his or her own level of understanding. When you sign this, you also note that your child understands and agrees to take part in this study according to his or her understanding.

Please print your child's name here: _____

Signing this consent form indicates that you have read this consent form (or have had it read to you), that your questions have been answered to your satisfaction, and that you voluntarily agree to participate in this research study. You will receive a copy of this signed consent form.

Subject Date

Legal Representative of Next of Kin Date Relationship to the subject

Investigator of Designee Obtaining Consent Date

Witness (if applicable) Date

Translator (if applicable) Date