

individuals, it is important that patients undergo annual screening for PAH via an ECHO.

#### If I have PAH, what should I do to improve my overall health?

There are several things you can do to improve your health if you have PAH. These include:

- Make sure you get sufficient rest.
- Keep your weight at a healthy level.
- If you smoke, stop immediately.
- Adopt a healthy diet that is high in fruits and vegetables and low in animal fats and hydrogenated oils.
- Keep active. You don't want to overdo things, but a modest amount of exercise can help. Consult with your physician to determine what is appropriate.
- Keep stress to a minimum.

PAH is a serious condition; however, PAH can usually be detected early and effective treatment begun in thalassemia patients in a clinical care setting with a team of experienced thalassemia experts.

The Thalassemia Clinical Research Network (TCRN) currently has an open trial to treat PAH with sildenafil or arginine. Patients with an ECHO that shows PAH are eligible.

For more information about PAH or the TCRN trial, please contact CAF at (800) 522-7222 or [info@cooleysanemia.org](mailto:info@cooleysanemia.org).



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*The information in this publication is for educational purposes only and is not intended to substitute for medical advice. You should not use this information to diagnose or treat a health problem or disease without consulting a qualified health care provider. The Cooley's Anemia Foundation strongly encourages you to consult your health care provider with any questions you may have regarding your condition.*

## Living with Thalassemia

Issues in Thalassemia Care

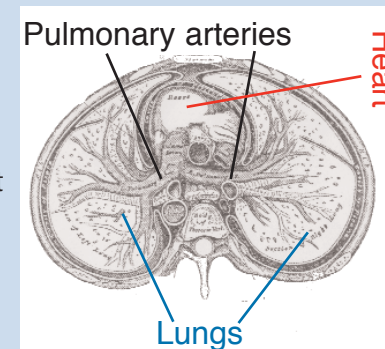
## Thalassemia and Pulmonary Hypertension

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#### What is pulmonary hypertension?

Pulmonary hypertension (often referred to as PAH or PHT) is a rare blood vessel disorder in which the pulmonary arteries (blood vessels in the lungs and leading from the heart to the lungs) get narrower. As a result, the blood pressure in these arteries rises above normal levels and, if the pressure is high enough, may become life threatening. Vessel narrowing can be due to:

- 1) muscles within the artery wall tightening,
- 2) pulmonary artery walls thickening, or
- 3) micro blood clots forming in the vessels causing blockage.



#### So high blood pressure and PAH are the same?

No. When we talk about "regular" hypertension (high blood pressure), we're usually talking about the reading you get when you strap an inflatable cuff to your arm and measure the blood flow there. In those cases, a normal reading is somewhere around 120/80.

But a normal blood pressure reading in the pulmonary arteries is much lower, around 25/15. It's possible to have both "regular" hypertension and PAH, but it's also possible to have a normal blood pressure reading and still have PAH.

#### What happens when the blood pressure in these pulmonary arteries gets too high?

The left side of the heart and the right side of the heart have different functions. Simply put, the left ventricle pumps blood all around the body; it has to work harder and is more "muscular" than the right. The right ventricle pumps the blood to the nearby lungs;

it doesn't need to exert as much effort, so it is less "muscular." In PAH, the pulmonary arteries, which lead from the right side of the heart to the lungs are narrowed. Therefore, the right ventricle of the heart must exert greater pressure to circulate blood through the lungs.

Working harder causes the right ventricle to get larger; after a period of time, it also becomes overstressed, which in turn causes it to become weaker. When this happens, you have a right ventricle that has become larger than it should be but, over time, has actually become weaker and is having a hard time pumping enough blood to the lungs. In some cases, this can lead to right heart failure.



*It's possible for a thalassemia patient to have a normal blood pressure reading and still have PAH*

#### What are some of the other complications?

In addition to an enlarged right ventricle and the danger of heart failure, PAH can lead to blood clotting or to fluid in the lungs. Both of these can be potentially dangerous.

#### What causes PAH?

The cause of PAH is not always known. In some instances, it may be the result of an underlying disease or condition, such as emphysema or bronchitis. PAH in individuals with thalassemia is thought to be related to conditions associated with thalassemia, such as a chronic anemia, hemolysis, the breakdown of blood cells and the increased tendency to form clots, particularly micro clots in the lungs. Splenectomy patients may have a greater risk for PAH. The reasons PAH occurs in some people with thalassemia are still being studied.

#### How does thalassemia complicate the risks associated with PAH?

Thalassemic individuals whose hemoglobin levels are consistently too low and are not being adequately transfused may be more susceptible to developing PAH. The bodies of patients with lower hemoglobin levels often try to compensate for the lack of hemoglobin via an increased heart rate which places additional effort on an already overworked heart.

Some studies have indicated that PAH may be somewhat more common in patients with thalassemia intermedia than in those with thalassemia major. However, all individuals with thalassemia should be aware that it can become an issue for them.

#### How do I know if I have PAH?

In non-thalassemia cases, symptoms of PAH include shortness of breath with minimal exertion, fatigue, chest pain, dizzy spells and fainting. If you experience these symptoms, you should bring them to the attention of your physician. S/he may then suggest one or more tests, ranging from a physical exam or an x-ray to an electrocardiogram or a cardiopulmonary exercise test.

However, many thalassemia patients with PAH are asymptomatic - they don't show any symptoms at all. For that reason, you should make sure that you undergo a complete comprehensive care examination in association with a qualified Thalassemia Center of Excellence every year; such regular thorough exams, which include an echocardiogram (also called ECHO), will often enable a physician to detect asymptomatic PAH in its early stages.

#### How is PAH diagnosed?

An ECHO can be used to assess right heart pressures. However, assessment of right heart pressure is not routinely performed on a standard ECHO. Therefore, patients should receive their annual ECHO at a Center of Excellence to ensure right heart pressures are measured. If right-sided pressures are elevated, your physician may suggest a cardiac catheterization to determine the exact pressure.

#### How is PAH treated?

In typical (non-thalassemic) cases, there are a number of options available for treatment, including blood thinners, calcium channel blockers, blood vessel dilators and sildenafil. (Sildenafil is a drug, popularly known as Viagra, that helps to open up and widen blood vessels.)

However, the awareness of the occurrence of PAH in thalassemia is still a relatively new development. The prognosis of PAH in thalassemia at this point is unclear, although several small scale studies have indicated that treatment with sildenafil is effective if the PAH is diagnosed promptly. Several prospective trials are ongoing which will provide physicians with a better understanding of the special characteristics of PAH in thalassemia, as well as its prognosis and treatment.

There is a great deal about PAH in thalassemia that still needs to be learned; however, because it is common in individuals with thalassemia, and because it is so often asymptomatic in these