Pleuropulmonary Blastoma - Childhood - Latest Research [1]

This section has been reviewed and approved by the Cancer.Net Editorial Board [2], 03/2016

ON THIS PAGE: You will read about the scientific research being done now to learn more about this type of tumor and how to treat it. To see other pages, use the menu.

Doctors are working to learn more about PPB, ways to prevent it, how to best treat it, and how to provide the best care to people diagnosed with this disease. The following areas of research may include new options for patients through clinical trials. Always talk with your child's doctor about the diagnostic and treatment options best for your child.

- **Drug combinations.** The experimental drug gefitinib (Iressa) is being tested in combination with irinotecan (Camptosar) and vincristine (Vincasar) to treat PPB in children. Cefixime (Suprax) and cefpodoxime (Vantin) are also added to the combination to reduce severe diarrhea for some patients.

- **Genetic causes of PPB**. As explained in Risk Factors [3], researchers are continuing to investigate the link between a genetic mutation to *DICER1* and familial PPB.

  Research into causes and treatment for a rare tumor like PPB requires collecting information from many hospitals. The International PPB Registry [4] is the largest such collection of information on PPB in the world. This registry has approval from the participating institutions' Institutional Review Boards [5] to ensure the protection of patients' privacy.

- **Treatment guidelines.** Currently, there are no large-scale organized treatment schedules for PPB because it is so rare. Individual doctors use their experience in treating similar conditions, such as soft-tissue sarcomas [6], to guide their PPB treatment recommendations. Plans are underway to create an international consortium of pediatric oncology specialists from around the world to consider treatment options and make recommendations for treating people with PPB.

- **Screening guidelines.** "Screening" is used to look for cancer before signs or symptoms appear.

  Doctors are looking at ways to screen children for PPB and other cancers related to *DICER1* genetic mutations. For PPB, screening guidelines will help doctors know when it's best use a CT scan to look for possible lung cysts or tumors, particularly for children under the age of 3. MRIs of the brain for children with the *DICER1* germline mutations are also being evaluated for screening guidelines.

  As outlined in Risk Factors, *DICER1* mutations are associated other cancers and tumors besides PPB. Below is a list of several of those cancers, along with current screening options for people already diagnosed with PPB. However, it is important to note that this is an area of active research. Options include:

    - Cystic nephroma:

      - Baseline kidney CT scan or ultrasound examination in a patient diagnosed with PPB. However, The negative predictive value [7] of a normal kidney CT or ultrasound examination for development of CN is not known.

      - In a patient of any age, especially those younger than 4 years, annual abdominal examination and monitoring for abdominal pain, swelling, or hematuria.

    - Thyroid gland neoplasia

      - Physical examination of the thyroid gland in a patient of any age

        - If the thryoid is not symmetrical and/or a nodule is detected, the patient may have a neck ultrasound examination. Results will indicate whether the doctor will monitor the patient or recommend a biopsy of the thyroid.

        - If no nodules are detected, continue annual physical examination. The doctor may also recommend repeating the thyroid ultrasound every 3 to 5 years.

    - Ultrasound examination of the neck may be recommended if the patient has previously received chemotherapy or as a baseline if the patient is going to have chemotherapy soon

    - Blood testing for thyroid functioning may be recommended, based on clinical signs and the patient's symptoms of an overactive or underactive thyroid gland.

- Ovarian stromal tumors

    - Physical examination for girls/women of any age for signs and symptoms of early development of puberty or virilization (development of male puberty characteristics), and/or masses in the abdomen or pelvis. If any are found, the doctor will recommend appropriate imaging and laboratory evaluations.

    - Imaging may include abdominal-pelvic ultrasound examination, MRI, or CT scan

    - Laboratory testing may include serum markers AFP, beta-HCG, LDH, inhibin A and B, estradiol, testosterone, CA125, and serum electrolytes including calcium levels.

      Note: There is no current recommendation for laboratory screening [8] in the absence of a mass or clinical findings of sex hormone excess.

    - For women and the parents of young girls with a *DICER1* germline [9] mutation, doctors will talk with you about the possible signs and symptoms of ovarian stromal tumors, such as abdominal distension, early puberty, amenorrhea (lack of menstruation), and signs of virilization.

- Ciliary body medulloepithelioma (CBME)

    - Physical examination of young children for this type of cancer, including measurement of visual acuity (how clear vision is)

        - Visual examination of the eyes and surrounding areas.

    - Botryoid embryonal rhabdomyosarcoma (ERMS).

        - In infants, children, and young adults when signs/symptoms of hematuria (blood in the urine) and/or abnormal vaginal bleeding are present, doctors may perform an endoscopic evaluation of the bladder and/or direct visualization of the cervix.

    - Nasal chondromesenchymal hamartoma (NCMH).

        - In patients of any age, the doctor will examine the body's various systems including respiratory and feeding difficulties, rhinorrhea, epistaxis, visual disturbances, and otitis media.

        - The doctor may recommend a nasal endoscopy if there are ophthalmologic signs (such as ophthalmoplegia, proptosis, ptosis, hypotropia, and enophthalmos) resulting from orbital involvement of the tumor

    - Pituitary blastoma**.**

        - Brain MRI for patients with a *DICER1* germline [9] pathogenic variant when there are signs of too much of the hormone cortisol

    - Pineoblastoma**.**

        - Brain MRI for patients with a *DICER1* germline [9] pathogenic variant with signs of increased intracranial pressure such as headache, full fontanel (soft spot in skull), vomiting, and lethargy, or other neurologic defects including upgaze paralysis (problems with eye movement) and nystagmus (rapid eye movements that the person can't control).

- **Palliative care.** Clinical trials are underway to find better ways of reducing symptoms and side effects of current PPB treatments to improve patients' comfort and quality of life.

Looking for More about Latest Research?

If you would like additional information about the latest areas of research regarding PPB, explore these related items that take you outside of this guide:

- To find clinical trials specific to your diagnosis, talk with your child's doctor or search online clinical trial databases now [10].

- Visit the website of the Conquer Cancer Foundation [11] to find out how to help support research for every cancer type. Please note this link takes you to a separate ASCO website.

The next section in this guide is Coping with Treatment. [12] *It offers some guidance in how to cope with the physical, emotional, and social changes that cancer and its treatment can bring. Or, use the menu to choose another section to continue reading this guide.*

Links
[1] http://www.cancer.net/cancer-types/pleuropulmonary-blastoma-childhood/latest-research
[2] http://www.cancer.net/about-us
[3] http://www.cancer.net/node/19551
[4] http://www.ppbregistry.org/
[5] http://www.cancer.net/node/24879
[6] http://www.cancer.net/node/31379
[7] http://www.ncbi.nlm.nih.gov/books/n/gene/glossary/def-item/negative-predictive-value/
[8] http://www.ncbi.nlm.nih.gov/books/n/gene/glossary/def-item/screening/
[9] http://www.ncbi.nlm.nih.gov/books/n/gene/glossary/def-item/germline/
[10] http://www.cancer.net/node/24878
[11] https://www.conquercancerfoundation.org/research-results
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