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Late Effects after a Pediatric Transplant Transitioning to Adult Care

Celebrating a Second Chance at Life Survivorship Symposium

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Late Effects of Hematopoietic Stem Cell Transplantation

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Hematopoietic stem cell transplants (HCST)/bone marrow transplants (BMT) are increasing in pediatrics

- Now over 3,000 per year
- Transplants are used for cancer
  - Leukemia, lymphoma, neuroblastoma
- Also for diseases of the blood
  - Immunodeficiencies
  - Sickle Cell Anemia
  - Hurler's Disease

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Young children are expected to live long into adulthood after transplant

- Children will grow substantially as they move into adulthood
  - Growth in size
  - Growth of organs/bones
  - Growth in intellectual function
- Parts of the transplant procedure can interfere with normal growth

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Topics For Discussion

- Basic principles to think about
- Specific examples
- The importance of a treatment summary
- Monitoring growth
- Striving to achieve independence

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Late Effects

- Any long-term difficulty experienced by a child from treatment
  - Can be physical
  - Can be intellectual (school performance)
  - Can be emotional
- If it impacts the child, it is important
- Research is relatively new (less than 40 years)
  - Still discovering new things as children grow into adults
  - Need to stay current with experts in the field

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Every component of the transplant can have an effect

- Chemotherapy
- Radiation
- Effects of other medications (steroids, tacrolimus, etc.)
- Illnesses during the transplant causing permanent organ damage
- Effects of the disease for which patient was transplanted
  - Hurler's disease (bone problems)
  - Adrenoleukodystrophy (adrenal insufficiency)
  - Cancer (damage from previous chemotherapy)

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Management after transplant is very complicated

- Some parts added together can make a problem worse e.g. radiation and chemotherapy
- Need help to pull everything together to make sense of the total picture

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Specific Examples: Thyroid Disease

- Thyroid is an organ in the neck that regulates energy
  - Radiation can damage it
    - Can also make it cancerous
  - Organ can get big and not function
    - More sleepy, hair thinning, retain weight
    - Can takes years to develop
  - Need regular blood tests (yearly)
    - Can develop slowly
    - Need to have neck examined to detect cancer early

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Specific Examples: Steroids

- Used to treat graft-versus-host disease (GVHD)
- Can cause diabetes, high blood pressure, and can thin bones
  - Can lead to chronic bone breaks
- Can lead to joint problems requiring replacements
- Need to monitor for bone thinning - bone density tests
- Need to optimize calcium and vitamin D intake to make the bones stronger
- Need exercise (weight bearing) to promote bone strength

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Specific Examples: School performance

- Many elements of the transplant can affect intellectual development
  - Radiation
  - Chemotherapy
  - Complications during the transplant course
    - Seizures
    - Strokes
- Children may initially do well in school
  - As they advance grades, work gets harder
- Regular neuropsychological testing
- Fight for services; often mandated by law
  - Do it early; may not get services in college if not approved in high school.
  - Won't reach their goals.

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Specific Examples: Fertility

- Different transplant procedures have different infertility risks
  - Non-myeloablative preserves fertility better than myeloablative
- Best to have discussion and plan before harvesting
  - Sperm banking and egg harvesting
- After the transplant, need to assess fertility potential
  - Sperm count for boys, AMH level
  - Consider banking and egg harvesting if there is still favorable results
  - May change if wait.
- Need to access centers with experience in this area
  - Costs
  - Explore resources that may provide financial assistance.

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Specific Examples: Sexual Development

- Children may experience delays or failure to advance into puberty
- This is different from fertility
  - Different parts of the sex organs
  - Can have a loss of fertility and have normal puberty
- Experienced providers will know if patient is not advancing on schedule
  - Pediatricians, Late Effects Providers, Endocrinologists
- May need hormone replacement if falls behind.
- Older children can also lose hormonal function
  - Early menopause, low testosterone
  - May also require hormone replacement therapy

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Specific Examples: Emotional Health

- Treatments may result in changes in physical appearance
  - Short stature, skin changes from graft-versus-host disease, chronic hair loss
  - Body Image
- Peer acceptance
  - Emotions may be out of sync with peers of comparable age
  - Physical limitations, chronic pain
- Acknowledge attribution to past therapy
  - Seek care from experienced providers

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Importance of a Treatment Summary

- A record of all of the complex medical information in a patient's history builds a picture of a patient's late effects risks.
  - Diagnosis
  - Complications of therapy (kidney damage, strokes, pneumonia)
  - Surgeries
  - Details of the transplant procedure
  - A life record
- Provides background information for all clinicians to provide thoughtful and informed management strategies.

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What should be in the treatment summary?

- Clinicians managing a patient's late effects can use the treatment summary to optimize wellness strategies
- Summaries of the issues that are relevant to your child
  - Guided by medical past
- Key elements to ask for:
  - "You are at risk for ……"
  - "As a consequence, you should be monitored in this way…"
  - "These are the signs to look for, and if you experience this, call your clinician….."
  - "These are the things you can do to promote your health and protect vulnerable organs"

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Make copies of your treatment summary

- The treatment summary provides a personal, portable tool that is *YOUR PROPERTY*
- Keep copies with you
  - Particularly if you move or change providers
- Recommendations embedded in your treatment summary can guide clinicians
  - Many lack experience in this field
  - You need to advocate for yourself and your child.

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Monitoring growth

- Young children will undergo many changes as they grow
- The age when they receive their therapy will often determine their vulnerability to later growth problems
- The youngest children are most susceptible to long-term injury from therapy.

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Children continue to grow

- Many organs continue to grow
  - Many organs may function fine when they are small, but may struggle as the child gets bigger.
- Growth is not restricted to physical organs
  - Intellectual growth
  - Emotional growth

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Regular Visits with a Late Effects Clinician are Important

- Regular screening tests
  - Many screening tests are quick, inexpensive tests identifying a problem
- Need to follow through with more sophisticated testing if the screening test shows something
  - The patient may not be showing obvious problems.
  - Early detection leads to early intervention and reduces the magnitude of a problem.

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Don't wait until the problems becomes obvious

- Child may become more sick
- May be harder to get services
- Windows of opportunity may close
  - Growth hormone treatments (bones may have already matured and no further growth is possible)
  - Fails class, may have difficulties getting into college
  - Menopause; can't harvest eggs to preserve fertility

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Striving to achieve independence – the end game

- Optimize health
- Optimize happiness
  - Address long standing emotional strains
- Optimize education
  - Long term occupations may be driven by late effects
    - Need to make educated decisions regarding the future
    - Don't choose unachievable goals
- Acquire a stable career/job
  - Benefits-insurance
    - Insurance coverage is essential immediately
    - Can't have gaps - health care needs are now, even if patient is young.

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Never too early to start developing a strategy

- Will invariably need to be "tweaked" as patient's personal late effects become evident
- The patient needs to be an active participant
- Will need to own the strategy once adulthood has been attained
  - Lifelong commitment
  - Develop plan and schedule when to take on more responsibility
    - Scheduling own appointments, visits alone, staying up to date with wellness strategy (diet, exercise, medications etc.)
- Adherence provides hope and promise for a bright future.

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Summary

- Pediatric survivors of HSCT/BMT can have diverse long-term problems with varying severity
- Can impact physical, intellectual and emotional state
- Treatment summaries can provide valuable information for both clinicians and patient families
- Families need to stay engaged with Late Effects clinicians to keep up to date in a constantly evolving area of medicine.

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Questions?

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