Short-ening stature: when grown up is too tall:

PHIL ZEITLER MD. PHD
DEPARTMENT OF ENDOCRINOLOGY
CHILDREN’S HOSPITAL COLORADO
UNIVERSITY OF COLORADO ANSCHUTZ MEDICAL CAMPUS
Disclosures

- I have no commercial disclosures relevant to this talk.
- I disclose that I believe that parents are the only ones who are in a position to know what is best for their children and their families.
A.D.

- 7 year 8 month old male with severe developmental delay, seizure disorder, cortical blindness following a neonatal stroke
- Family read about and was interested in growth attenuation therapy, but were unable to identify a willing provider at their regional Children’s Hospital
- AD requires full care. Diapered, drinks from a sippy cup if it is held for him and eats pureed food. Unable to help with transfers - will lift his leg up to be in lap.
- Unable to communicate but will reach for sippy cup or mom's hands. No response to questions, no sign language
- Goes to MR/DD program and has 40 hour a week nurse and home health assistant 35 hours a week. Family has hoyer lift and wheelchair van
- Lives with mom, dad, two daughters - 10 and 5
- He likes to “cuddle, bathe and eat”
Family concerned he will be restricted from participating in regular activities if he grows to his expected height. He enjoys activities such as hiking etc. Mom can still carry him on her back, but doesn’t know how much longer.

Two nanny’s have had fractures from lifting him and he has had multiple falls when he has been lifted and carried. He has fallen on mom when she is carrying him and has fallen on her in the bathtub.

“The hoyer lift doesn’t go into the bathtub” and “You can’t take a hoyer lift with you when you leave the house”

When he was younger, they were unsure where he would be functionally and would not have intervened, but they are now more aware of what he will probably do and won't do.

Pulmonary supportive of attenuation due to marginal respiratory status as he is getting bigger.
A.D.

- Family goal to make it possible for him to stay with his family.
- He recognizes family faces and voices and is calmer at home. The family does not feel that he would do well in another residential setting or an institution, even if quality institutions existed.
  - When mom was growing up she had a great uncle that her grandmother cared for until she got too old. Then he was put into a family home but the host family was into drugs and the great uncle was poorly cared for – the family finally got him out.
- They do not feel that stature has any meaning to him and they believe that he would choose to stay with the family over any other choice if he could make this decision.
- "Options are never good you have to pick the best one for the family."
Reviewed potential risks and unknown benefits

Ethics consult not formally obtained because CHCO ethics committee previously determined that

- there are no fundamental ethical issues in growth attenuation
- the wishes of the family should be followed once it is determined that they are acting according to their perception of the best interests of the child

Baseline laboratory evaluation obtained to exclude contraindications

Family elected to proceed

- Estradiol 2 mg a day and increase by 2 mg a week until 10 mg a day
- Treat until near total fusion of epiphyses
How it all started: Ashley

Article
October 2006

Attenuating Growth in Children With Profound Developmental Disability
A New Approach to an Old Dilemma

Daniel F. Gunther, MD, MA; Douglas S. Diekema, MD, MPH

Editorial
October 2006

Growth Attenuation
A Diminutive Solution to a Daunting Problem

Jeffrey P. Brosco, MD, PhD; Chris Feudtner, MD, PhD, MPH
• Growth attenuation therapy administered to the little girl known as Ashley is an affront to her human dignity

• It is the duty of both caregivers and the hallmark of a progressive, civilized society to provide the means by which all of us can reach our full human potential

• This approach to easing the hardship of caring for a child with disabilities makes the child the problem, and by doing so, makes it acceptable for well-meaning people to deny the essential humanity of people with disabilities in the course of caring for them
Joel Frader (Professor of Pediatrics/Medical Humanities and Bioethics at Northwestern University Feinberg School of Medicine):

- Parents and other care providers for entirely dependent patients face physical challenges when the patients grow large.
- Difficult to argue that these patients benefit merely from reaching their genetic height potential.
- Reducing the demands and stress on caregivers serves the interests of the patients.
- Our society provides insufficient support for persons with disabilities. A more generous society would make sure caregivers have assistance devices and other help necessary.
- However, in the absence of an adequate system, the measures taken to limit growth seem reasonable and well within the scope of caring parents' authority to undertake.
Norman Fost (Professor of Pediatrics and Bioethics; Director of the program in Bioethics and Vice Chair, Department of Medical History and Bioethics, University of Wisconsin Medical School)

Compelling arguments that limiting Ashley's growth is likely to be in her interests, as well as the parents' interests, with low risks.

Some argue that the measures used to limit her growth "miss the point," which is the lack of social support which might allow the family to care for her at home even if she grew to normal adult size; e.g., by providing mechanical devices to assist in lifting her. I think this misses the point, which is to try to maximize Ashley's interests, or more precisely, to maximize the ratio of benefit to burden.

Her interests will be better served by limiting her size, regardless of whether devices or supportive services are available. She is more likely to be brought on trips and to have more pleasurable experiences.
Benjamin Wilfond (Director, Treuman Katz Center for Pediatric Bioethics at Seattle Children's Hospital; Professor of Bioethics, Department of Pediatrics, University of Wisconsin):

- The issue of the impact of size on encouraging "mobility" would generally be true whether the care is provided by family members or by other care givers. So even having better services would (not) fully address the issue.

- While the parents are doing their best to anticipate how difficult it might be to manage their child as she got larger, their prediction is speculative.

- Some issues that seem insurmountable when looking forward become more manageable when the situation is at hand.

- But it doesn't always go that way

- I do think that we should respect parents' wishes to make such decisions, particularly after such prolonged engagement and discussion.
Our analysis suggests that growth attenuation is an innovative and sufficiently safe therapy that offers the possibility of an improved quality of life for nonambulatory children with profound cognitive disability and their families.

Pediatricians and other care providers should include discussion of these options as part of anticipatory guidance around the age of 3 years so that, if elected, potential clinically meaningful benefits of growth-attenuation therapy can be realized.

Because of the publicity and debate surrounding the first reported case, ethics consultation is recommended.
Unjustifiable Non-therapy: A response to Gunther & Diekma (2006), and to the issue of growth attenuation for young people on the basis of disability from The Board of Directors of the American Association on Intellectual and Developmental Disabilities. (2012)

- No studies have found that the interventions lead to an increase in the amount of time a young person remains in a family home.
- No studies have tracked any other outcomes, including medical complications, perceived self-efficacy, impacts to the medical providers, or any other downstream effects. We are shocked that they would have the temerity to suggest such a therapy without a single study to support their claims.
- Medical history has had too many prior examples of misguided scientific “advances” that turned out not to actually be beneficial.
- Abundant evidence that all children are able to learn and that the cognitive capabilities of children with severe motor impairments can be grossly underestimated, and although we do not pretend to be as familiar with this situation as the treating physicians, we think that extant case histories of people with cerebral palsy incorrectly diagnosed as having profound intellectual disability should be recognized and discussed.
The precedent setting potential for growth attenuation can lead to misuse. The intervention does not address the core problems faced by families and our society regarding services and supports to affected individuals. Nothing in the disabilities rights movement to justify imposing constraints on anyone’s development. Assent was not obtained from this child. Ever-expanding options for in-home supports. Under our law, parents are vested with the responsibility for making health care decisions for their minor children, but parental prerogatives are not absolute. Growth attenuation should not be included as an option. It should be perfectly acceptable to prescribe whatever dosage of psychoactive medication would be necessary. Medical practice for an individual can rapidly degenerate if the anxieties of the parents regarding an as-yet unclear future issues replace the medical best interest of the child as the primary focus. We are stunned and outraged by the very fact that the relative merits of growth attenuation could still be, in 2012, a topic for serious discussion.
This benefits the parents, not the child

- Some of the interventions will benefit the parents in important ways. That alone does not make the interventions wrong. If the interventions will also benefit the patient, there is a congruence of interests, not a conflict of interests.

Growth attenuation infantilizes the patient

- Reducing Ashley’s adult height to 4'6” does not infantilize her. While a height of 4’6” is short, it is well above the height of infants and young children.

- She will age normally, and have the same hormonal fluctuations that other adolescent and adult women experience.

- To anyone not familiar with her history, a 30-year-old Ashley will appear as any 30-year-old adult with the same disability.

- Short adults and conditions that may result in extreme short stature, such as achondroplasia or hypopituitarism, do not look like children; they are unmistakably adults, however short.
This makes the patient a freak

- A height of 54-61” is not “freakishly” short
- Individuals with severe short stature due to skeletal dysplasia are not “freaks” and the concerns raised by the disability community do not address this paradox
- The patient will age normally
- The patient’s mental development, a result of the underlying condition, will be the feature that remains the most “child-like” and the most unlike other individuals

Violation of dignity

- While dignity seems to be connected to providing the means to reach one’s full human potential, it is not clear how the medical interventions will keep them from reaching the same potential as prior to the interventions, other than the potential for normal height
Patients have a right to physical integrity and a right to grow and develop

- A right to physical integrity does not preclude us from violating someone’s physical integrity with their permission, or the permission of an appropriate surrogate, when there is a commensurate benefit – removal of tumors, tonsils, and appendices, or insertion of gastrostomy and tracheostomy tubes, and performing osteotomies

- A “right to grow” implies that normal height is an end in itself. The goal of treatment of short stature is not to achieve a measurement that is in the normal range, but to produce a size that is functional within the parameters of the individual’s circumstance

- Would we recommend/require growth hormone therapy were the child destined to be short? If a “right to grow” were taken seriously, critics would to argue that parents should be charged with neglect if they refused to consent to such treatment
A digression: Why is making someone shorter upsetting but not making them taller?

- We shouldn’t limit stature because it is preventing the full development of the child
  - Stature = degree of development, esteem, and success.
  - Limiting height is equivalent to limiting opportunities
  - By extrapolation, shorter people are less desirable than taller people.
- There is no hesitation about the use of GH to make medically complex children taller
- It would be better to place the child in an institution that “can care for them” rather than do something to make them smaller
- The objections to GA reveal the profound height bias of our culture
  - The objections of the disability community avoid addressing the implications of the position for those with severe skeletal dysplasias and suggest that they are less capable or developed.
As Endocrinologists, we do our best to approach height not as a “good” in itself, but tied to function.

- Criteria for treatment of short stature are based on arguments about functional capacity.
- The argument for making short people taller is that they are not optimal for their environment – can’t reach cabinets, drive cars etc, less socially desirable.

- Doesn’t the optimal height for someone depend on the social requirements of their environment?
- Why should we expect the same height be optimal for someone in a wheelchair or for someone who is dependent on others to carry them as for someone who ambulates - unless we believe that height itself is a good?
Failure to respect autonomy

- The patient’s condition, not the treatments robbed them of autonomy
- Restricting parental decisions would decrease freedom by removing certain options from patients
- Patients, when they cannot exercise their right to choose, have a right to have them exercised by a surrogate
- Patients likely can not appreciate, benefit from, or be aware of a “right” to have “normal size. On the other hand, patients do have an interest in being loved, cared for, and valued
- Patients have a right to be treated in a way that is appropriate to their circumstances and that recognizes how their needs, including the promotion of an optimal quality of life, might differ from others
This is not natural…playing god

- Rejecting unnatural treatments would preclude most medical practice considered standard in the care of these children.
- If we “play God” in offering certain interventions, we also “play God” when we decide not to intervene. A decision to “do nothing” will affect the outcome of their life as surely as a decision to “do something.”

Society needs to change, not the child

- Society’s failure to respond to the needs of those who care for a child with disability does not preclude an intervention that has the potential to improve the quality of life for the child and the family.
- Families do not live in an ideal world.
- Many patients would be better off if more resources were available, but it does not follow that they should be prohibited from accessing treatments, even if they would be second best in an ideal world.
Another digression: Are institutions a reasonable option?
There is no medical indication for these interventions

- The tools of medicine are used commonly to treat disorders or conditions that are commonly defined as social - laser treatment for facial hemangiomas, braces for crooked teeth, drug prescriptions for acne

- Drugs to improve fertility, ADHD, many psychiatric disorders, severe phobias, erectile dysfunction, growth hormone treatment, and sleep disorders could all be considered social rather than medical, disorders
There is potential for misuse or abuse if applied to other children

- The slippery slope is sometimes a path to something of value that can only be attained by risking the slope
- Refusing to offer something of benefit to an individual because of the potential for a slippery slope may ultimately harm that individual
- Many technologies are misapplied at some point. The wheel has made possible the premature deaths of millions, but few would argue against its use
- It is problematic to withhold a potentially beneficial treatment from a patient on the grounds that it might be misused on some future person. A physician has a duty to promote the welfare of the patient in front of them at the moment
- “The good mountain climber does not simply deny the risk of the slippery slope, but rather recognizes the risk, trains him- or herself to be aware of the dangers, places protection against those risks, uses caution, and is willing to turn around when the dangers appear to be unavoidable or the risks unacceptable”
**Key Findings**

Number of respondents who have been asked about GAT for a child with SPCD: 98

Number of respondents who have prescribed GAT for a child with SPCD: 32

Number of children with SPCD treated (minimum calculation): 65

Number of respondents who have withheld treatment of precocious puberty to attenuate growth in a child with SPCD: 130

Percent of respondents who obtained ethics consultation for GAT of children with SPCD: 34%

Percent of respondents who obtained ethics consultation for withholding treatment of precocious puberty in order to attenuate growth in children with SPCD: 5%

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**Respondents' Attitudes About GAT**

- "Growth attenuation therapy is sometimes appropriate."
- "GAT should be actively offered for cases of severe physical and cognitive disability, not just when raised by a parent."

**Disagree**

- 33, 15%

**Agree**

- 190, 85%

**Disagree**

- 135, 75%

**Agree**

- 44, 25%

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**Reasons respondents did NOT prescribe GAT in children with severe physical & cognitive disability**

1. Family decided against (63%)
2. Concern about side effects (43%)
3. Legal concerns (13%)
   - Concern about publicity (13%)
4. Personal conviction or beliefs (10%)
5. Ethics consultation recommendation (7%)
Our first case: March 2016

The New York Times Magazine

Should Parents of Children With Severe Disabilities Be Allowed to Stop Their Growth?
A.D.
CHCO Experience results to date: change in bone age/year (n=35)
CHCO Experience results to date: change in PAH after ~ 1 year (n=25)
CHCO Experience results to date: change in PAH after ~2-4 years
CHCO Experience results to date: change from baseline

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CHCO Experience results to date: a few kids who have reached final height

Average 6.4 inches
Range 2-11 inches
Safety outcomes

- Gynecomastia common in males
- Menstrual bleeding common in girls (placed on progesterone)
- Protein S and Protein C commonly fall – no clots
- Moodiness and some change in behavior common
- No clear change in seizure pattern reported
- 1 case of increased skin fragility
- No fractures
- Prolactin elevation common, particularly among peripubertal age individuals
Resistance to assisting with growth attenuation remains widespread, despite surveys suggesting that PES members are personally supportive.

Resistance by providers and hospitals appears to stem from a vague sense of ethical controversy.

Bioethicists, in general, consider GAT to be ethically acceptable and within the realm of parents’ rights to make decisions for their children.

Resistance has come nearly exclusively from the disability community, which rightly views GAT in the context of a long-history of mistreatment.

Resistance to growth attenuation conflates height and development and probably reveals societal height bias.
As endocrinologists, we recognize that height is not a “good” in itself but can only be understood within the concept of functional height.

There is no *a priori* reason why optimal functional height would be the same for ambulatory and non-ambulatory individuals.

We have the same duty to support functional height attainment in non-ambulatory as ambulatory patients.

Limited data on outcomes suggest:

- Height loss with high dose estradiol is in the range of 5 inches, but with a large range.
  - Interestingly, this is quite similar to outcomes of treatment of tall stature in girls in the past.
- The taller the MPH and initial PAH, the greater the height loss.
- The earlier the treatment, the greater the height loss.
Conclusions

- Growth attenuation is an ethically defensible treatment to promote optimal functional height for carefully chosen individuals.

- The choice to undertake growth attenuation should be cognizant of the history of the therapy and the sensitivities of the disability community, but providers should not be paralyzed by a vague sense of ethical controversy.

- Evidence-based discussion with your institutional ethics committee is appropriate to establish policies, with individual ethic consultation when needed.

- The outcomes of growth attenuation should be tracked to provide better data and we should have the courage to be public enough to start a registry.

- For now, whether the expected loss of height justifies the risks of therapy, albeit low, should be a frank and complete discussion with the family.
Thank you!