

Howard Williams Oration

RACP Congress

Adelaide

May 2016

Professor Dinah Reddihough

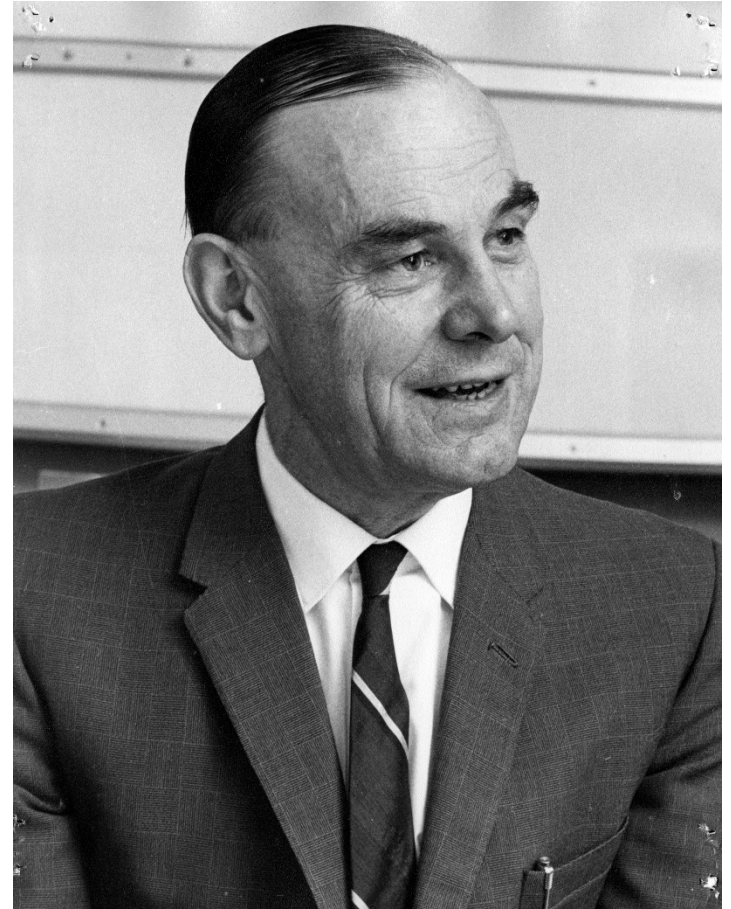
Melbourne
Children's

Excellence in
clinical care,
research and
education



Howard William's Legacy

- “Health of children basis for health of the nation”
- Leaders in paediatrics should be trained in
 - High quality research
 - Exemplary clinical care



Howard William's Legacy

- Respiratory Medicine, epidemiology of childhood asthma
- The Brunswick Study
 - “new morbidity”
 - Major health problems in children not physical but behavioural stemming from
 - Family dysfunction
 - Adverse life events
 - Maternal depression

Howard William's contributions

- He never lost sight of the importance of the clinical contact between child and doctor
- Took account of familial and social factors
- Magnificent clinical teacher

What lessons can we learn from Howard Williams when we think about children with disabilities?

Rates of cerebral palsy

34,000 Australians

1.5 - 2 / 1000 live births



What do we want for children with cerebral palsy in Australia?

The best possible:

- Inclusion
- Participation
- Quality of life for all children with cerebral palsy and their families

Currently some receive good treatment. For others:

- There is premature death
- Postural deformities
- Poor nutrition
- No diagnosis being made
- Families being poorly informed and supported

We remain at the crossroads....

- More information about risk factors and causes
- More evidence about the effectiveness of interventions
- Further skills in how to engage families and respond to their needs



Why did it happen?

Risk factors

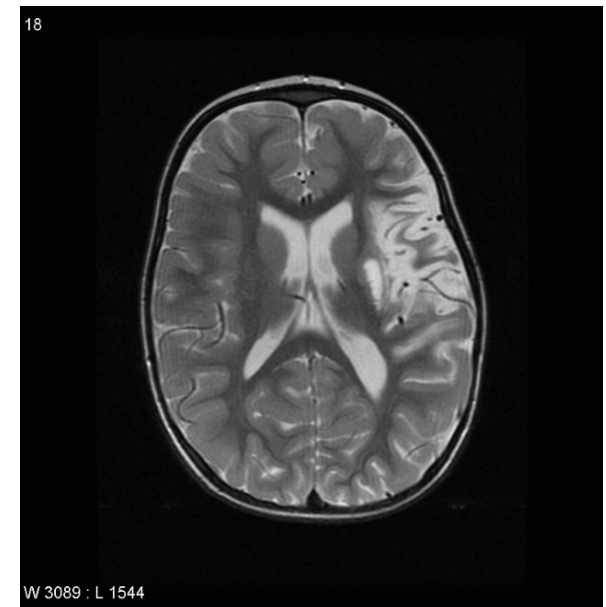
- Prematurity
- Low birth weight
- Multiparity
- Fetal exposure to maternal infections and inflammation

But the risk factors and causes for many children remains unknown

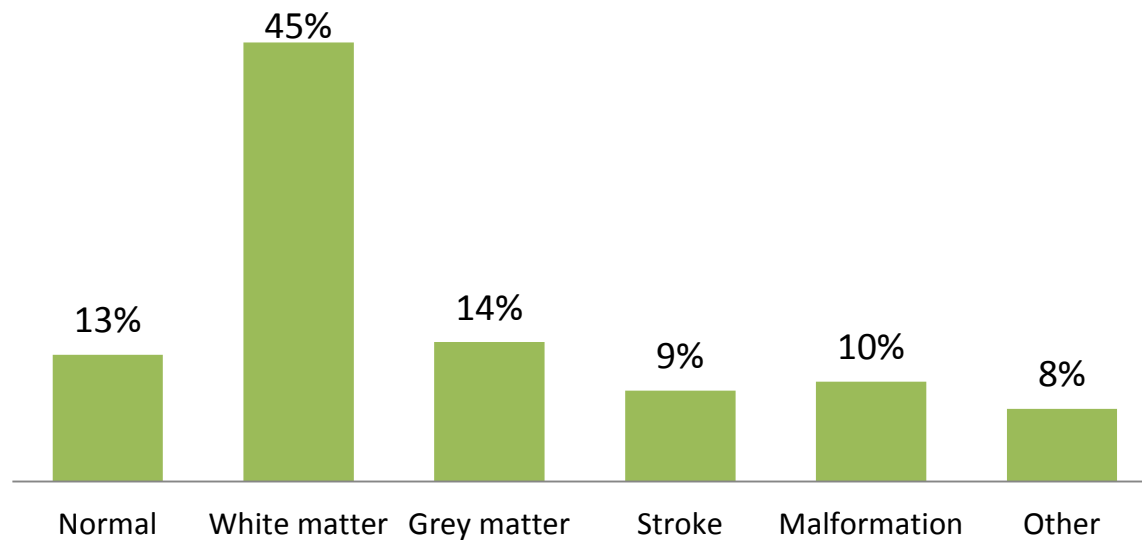
Advances in diagnosis

2004 – American Academy of Neurology

- All children with cerebral palsy of unknown aetiology should undergo neuroimaging, with MRI being the preferred investigation
- Will confirm neuropathology in about 80%



Patterns of brain injury



Further investigation

American Academy of Neurology recommends

- Genetic and / or metabolic investigations when neuroimaging fails to establish a specific abnormality or when there are atypical features
- Treatable cause may be found eg dopa responsive dystonia or a neurodegenerative condition may be discovered
 - diagnosis of cerebral palsy then excluded

We remain at the crossroads....

Do all children in Australia get a diagnostic work up to establish if the cause can be determined?

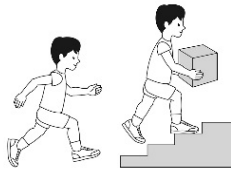
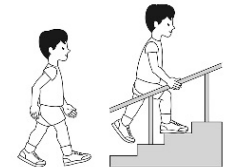
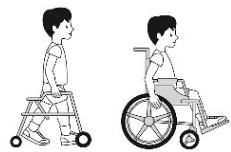
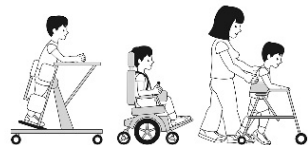

Or does care vary by location / availability of resources?



Assessment tools – the GMFCS

- Reliable across users
- Cited well over 1000 times in the literature
- Translated into at least 25 languages

GMFCS E & R between 6th and 12th birthday: Descriptors and illustrations

	<p>GMFCS Level I</p> <p>Children walk at home, school, outdoors and in the community. They can climb stairs without the use of a railing. Children perform gross motor skills such as running and jumping, but speed, balance and coordination are limited.</p>
	<p>GMFCS Level II</p> <p>Children walk in most settings and climb stairs holding onto a railing. They may experience difficulty walking long distances and balancing on uneven terrain, inclines, in crowded areas or confined spaces. Children may walk with physical assistance, a hand-held mobility device or used wheeled mobility over long distances. Children have only minimal ability to perform gross motor skills such as running and jumping.</p>
	<p>GMFCS Level III</p> <p>Children walk using a hand-held mobility device in most indoor settings. They may climb stairs holding onto a railing with supervision or assistance. Children use wheeled mobility when traveling long distances and may self-propel for shorter distances.</p>
	<p>GMFCS Level IV</p> <p>Children use methods of mobility that require physical assistance or powered mobility in most settings. They may walk for short distances at home with physical assistance or use powered mobility or a body support walker when positioned. At school, outdoors and in the community children are transported in a manual wheelchair or use powered mobility.</p>
	<p>GMFCS Level V</p> <p>Children are transported in a manual wheelchair in all settings. Children are limited in their ability to maintain antigravity head and trunk postures and control leg and arm movements.</p>

Assessment tools



Manual Ability Classification System for Children with Cerebral Palsy 4-18 years

MACS classifies how children with cerebral palsy use their hands to handle objects in daily activities.

- MACS describes how children usually use their hands to handle objects in the home, school, and community settings (what they do), rather than what is known to be their best capacity.
- In order to obtain knowledge about how a child handles various everyday objects, it is necessary to ask someone who knows the child well, rather than through a specific test.
- The objects the child handles should be considered from an age-related perspective.
- MACS classify a child's overall ability to handle objects, not each hand separately.

2005, updated 2010



Communication Function Classification System (CFCS) for Individuals with Cerebral Palsy



Purpose

The **purpose** of the CFCS is to classify the **everyday communication performance** of an individual with cerebral palsy into one of five levels. The CFCS focuses on activity and participation levels as described in the World Health Organization's (WHO) International Classification of Functioning, Disability, and Health (ICF).

User Instructions

A parent, caregiver, and/or a professional who is familiar with the person's communication selects the level of communication performance. Adults and adolescents with cerebral palsy may also classify their communication performance. The **overall effectiveness** of the communication performance **should be based on how they usually take part in everyday situations requiring communication**, rather than their best capacity. These everyday situations may occur in home, school, and the community.

Some communication may be difficult to classify if performance falls across more than one level. In those cases, choose the level that **most closely describes** the person's usual performance in the **most settings**. Do not consider the individual's perceived capacity, cognition, and/or motivation when selecting a level.

Definitions

Communication occurs when a **sender** transmits a message **and** a **receiver** understands the message. An **effective communicator** independently **alternates as a sender and a receiver** regardless of the demands of a conversation, including settings (e.g., community, school, work, home), conversational partners, and topics.

All methods of communication performance are considered in determining the CFCS level. These include the use of speech, gestures, behaviors, eye gaze, facial expressions, and augmentative and alternative communication (AAC). AAC systems include (but are not limited to) manual sign, pictures, communication boards, communication books, and talking devices – sometimes called voice output communication aids (VOCAs) or speech generating devices (SGDs).

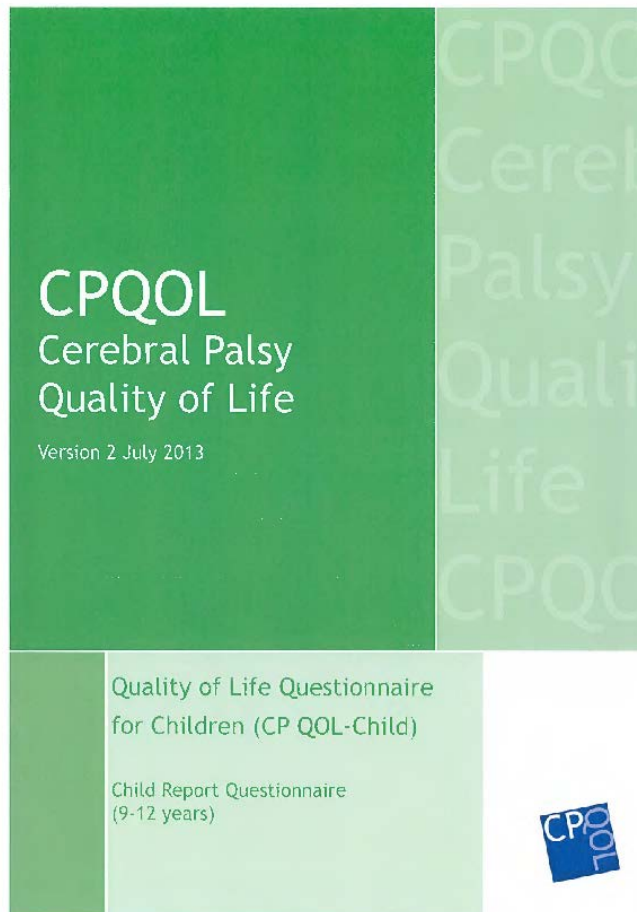
Distinctions between the levels are based on the performance of **sender and receiver roles**, the **pace of communication**, and the **type of conversational partner**. The following definitions should be kept in mind when using this classification system.

Effective senders and receivers shift quickly and easily between transmitting and understanding messages. To clarify or repair misunderstandings, the **effective sender and receiver** may use or request strategies such as repeating, rephrasing, simplifying, and/or expanding the message. To speed up communication exchanges, especially when using AAC, an effective sender may appropriately decide to use less grammatically correct messages by leaving out or shortening words with familiar communication partners.

A **comfortable pace** of communication refers to how quickly and easily the person can understand and convey messages. A comfortable pace occurs with few communication breakdowns and little wait time between communication turns.

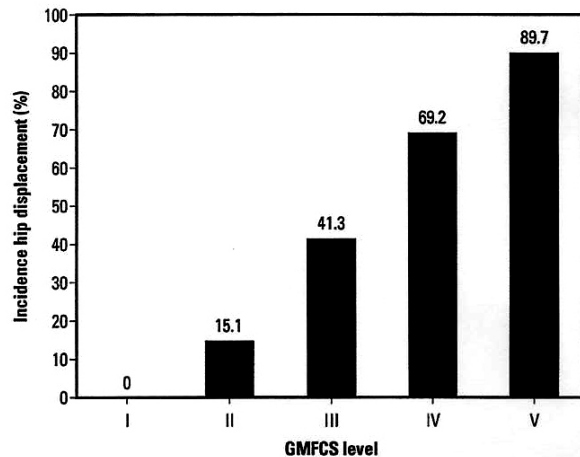
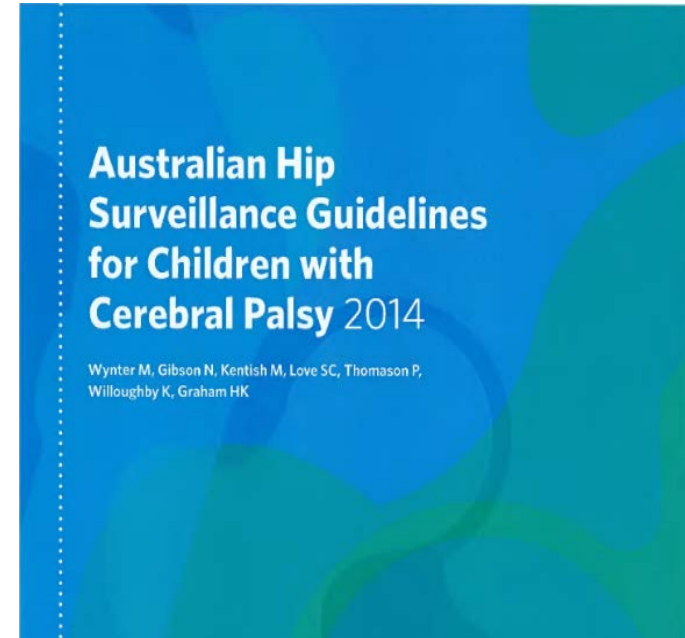
Unfamiliar conversational partners are strangers or acquaintances who only occasionally communicate with the person. **Familiar conversational partners** such as relatives, caregivers, and friends may be able to communicate more effectively with the person because of previous knowledge and personal experiences.

Cerebral Palsy Quality of Life for Children



- Translated into 24 languages
- Shortened version being developed to make more clinically relevant

Hip Surveillance Guidelines



Incidence of hip displacement (a migration percentage of >30%) according to the Gross Motor Function Classification System (GMFCS) level.

<https://ausacpdm.org.au/professionals/hip-surveillance/australian-hip-surveillance-guidelines/>

We remain at the crossroads....

Do all children in Australia get appropriate assessment and surveillance tools?



Or does care vary by location / availability of resources?

What needs to be done to ensure that every Australian child receives appropriate surveillance?





Follow up program for people with cerebral palsy in Sweden

- Continuous and standardised assessments during childhood
- Regular physical assessments by an occupational and a physiotherapist
- Demonstrated to prevent hip dislocation, and reduce the number of children with severe contractures and scoliosis
- 95% of families participate

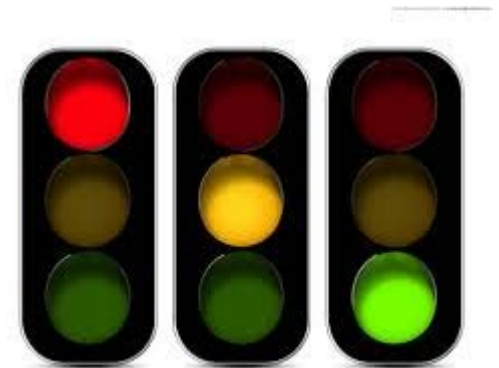
Australian version



- In Australia, a modified form of CPUP has been developed, named “CP-Check-UP™”
- Currently being trialled as part of an NHMRC Partnership project to determine if therapists will use the tool and input data on regular basis
- Implementation will take time and co-operation across all sectors
- Can we introduce uniform surveillance across Australia as in Sweden?

Management

- Evidence based practice first appeared 1991
- AACPDm declared in 2013 that “the care that children receive is disproportionately dictated by where they live, who they see, and how their care is financed, rather than what is warranted by their condition.... The translation of the best science and the best “art” into practice for all children is still more hope than reality”



Approach to management

- Health and secondary prevention approaches
 - Management of the child's health and comorbidities
 - Prevent or lessen the natural history, for example, treatment of contractures
- Child-active rehabilitation approaches / therapy
- Compensatory and environmental approaches

Management of health issues and related co-morbidities

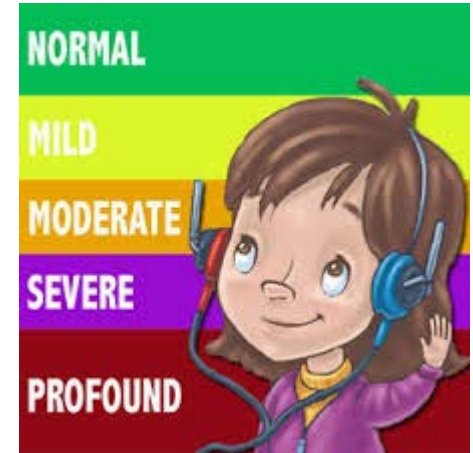
Do all children get their hearing assessed?

Do all children get their vision assessed?

Do we enquire about immunisation history?

What about regular dental care?

Are boys screened for undescended testes?



Management of health issues and related co-morbidities

Epilepsy

Better treatment with more medications available

- All seizure types are seen
- Some types of epilepsy are a direct consequence of the underlying brain injury and may persist throughout life.
- Other types of epilepsy are not directly related to a child's cerebral palsy eg benign childhood epilepsy with centrotemporal spikes (Rolandic epilepsy)

Nutrition and feeding

Team approach required

Is a gastrostomy beneficial?

Sullivan et al

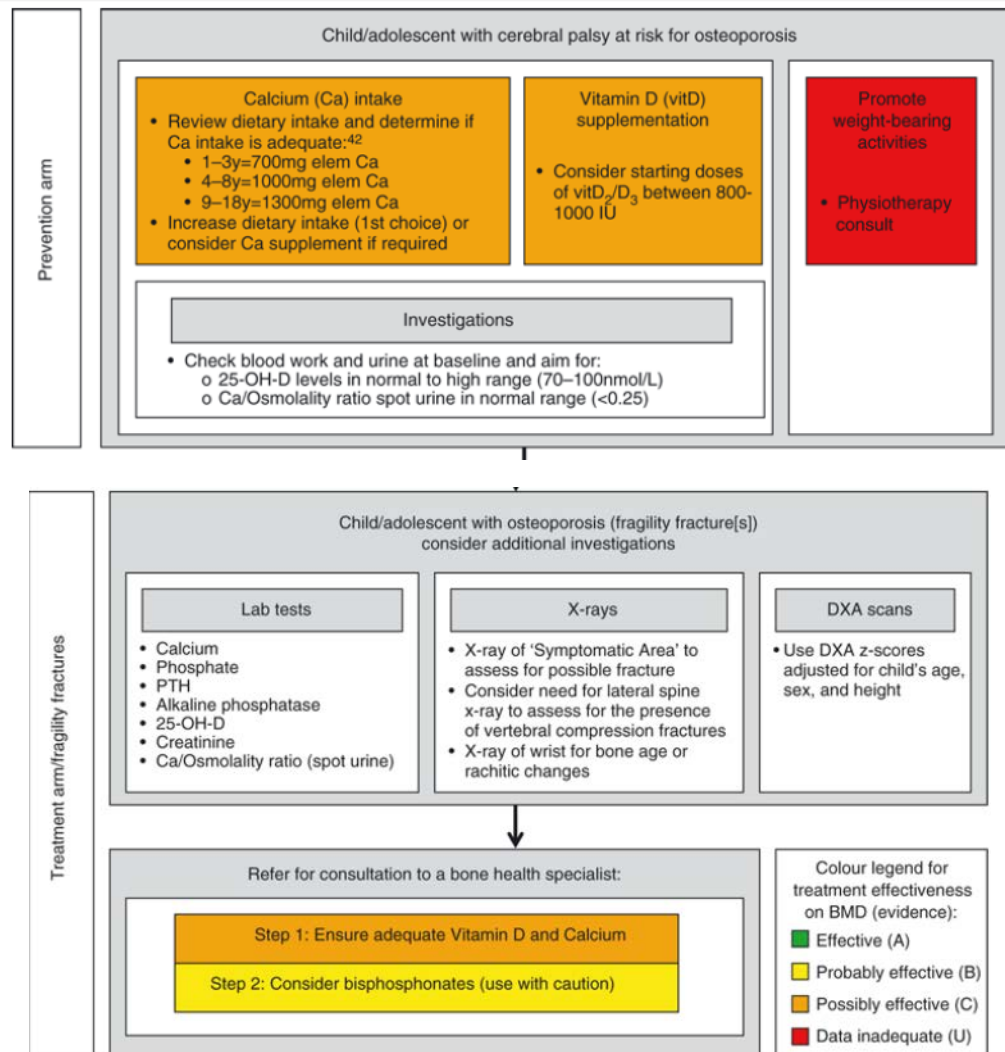
- children with severe cerebral palsy followed for 6-12 months
- significant improvements in weight and subcutaneous fat deposition
- almost all parents reported improvement in their child's health
- decreased time spent feeding with significant improvements in quality of life for caregivers.

Cochrane review 2010 - no randomised trial

Clinical Guideline for Osteoporosis

Are these guidelines followed?

More information about the use of bisphosphonates



Other gaps in knowledge

- How best to manage and prevent recurrent chest infections and lung disease
- Optimal treatments for saliva control
- Appropriate management of behavioural / psychological issues

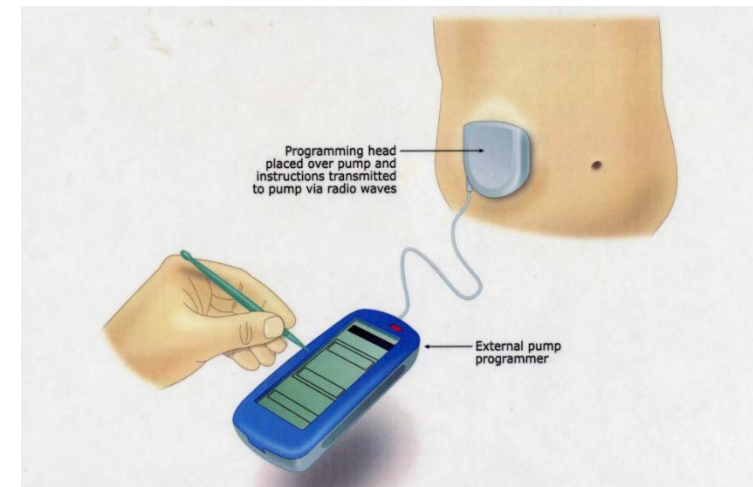
Prevent or lessen the natural history, for example, spasticity management

Role of oral baclofen for spasticity

- Cochrane Review identified six randomized controlled trials involving a total of 130 patients
- Studies show a great variability in motor classification, dosage of baclofen, and outcome measures
- Conflicting evidence on the effectiveness of oral baclofen in reducing muscle tone or improving motor function or activity level
- Overall methodological quality of the studies was low, with risk of bias, inconsistency of results, unpowered sample size
- The role of oral baclofen in spasticity has still not been fully evaluated.

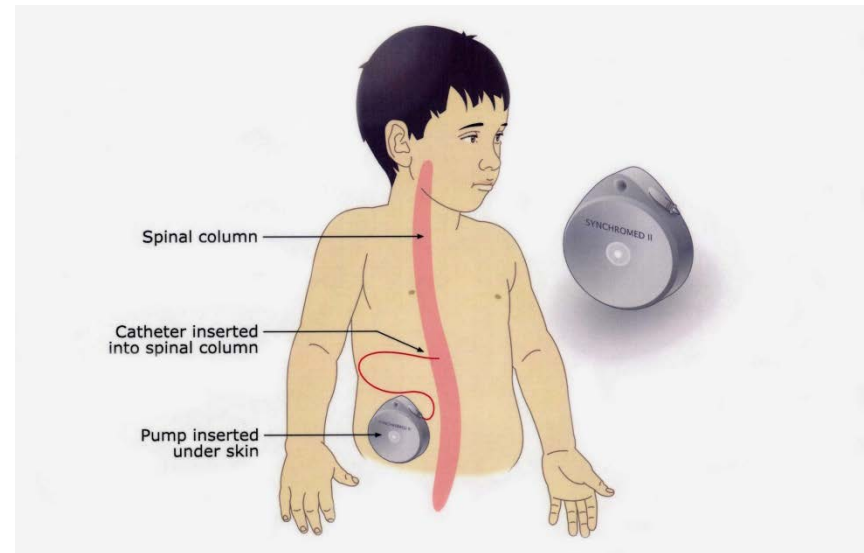
Role of intrathecal baclofen

- Used for children with severe spasticity / dystonia interfering with care, comfort and quality of life
- Intrathecal baclofen is delivered directly to its site of effect
- Pump readable through an external programmer



Role of intrathecal baclofen

- Evidence of effectiveness for treating spasticity, improving comfort, ease of care and quality of life is needed.
- Studies constrained by small sample sizes / methodological issues.
- Need to explore
 - questions regarding the subsequent need for orthopaedic surgery
 - the safety and the economic implications of this treatment



Other treatments

- What about other drugs for spasticity?
- What about drugs for dystonia eg Benzhexol?
- What is the value of hand splinting?
- When should children be referred for scoliosis management?
- Is early bony surgery for displaced hips better than early soft tissue releases?

Many questions remain unanswered

We remain at the crossroads....

Do all children in Australia get the most appropriate treatment from the evidence available?



How can research best be undertaken and funded to answer the numerous unanswered questions?



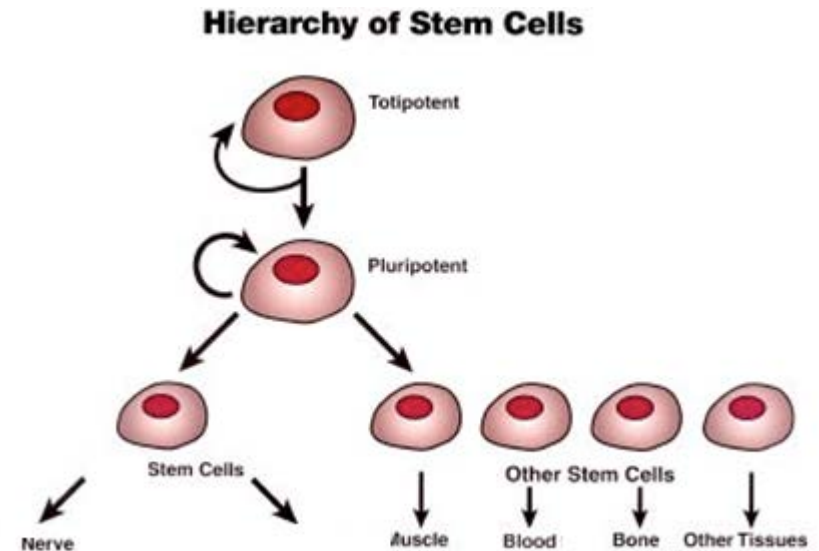
Effective interventions

- Goal-directed training – child-active structured training in self-care tasks eg dressing, designed so task is meaningful for child
- Bimanual training for hemiplegia (30 – 60 hrs of therapy over 6-8 weeks)
- Constraint-induced movement therapy for hemiplegia (30 – 60 hrs of therapy over 6-8 weeks)
- Strength training
- More evidence needed about a range of therapies including Bobath and Conductive Education

What about radical new treatments such as stem cells?

Pre clinical research:

- Probably not by replacing lost cells
- Perhaps by paracrine effects, by releasing cytokines that can circulate in or to the brain
- These may help the body to:
 - re-wire damaged regions of the brain
 - make available the native neural stem cells of the brain
 - improve blood vessels in the brain with angiogenesis
 - (modulate inflammation, reduce secondary cell death after acute injury)



Role of stem cells

Clinical research:

- What are the risks of treatment?
- What are the outcome measures needed to determine benefit: motor, communication, cognitive?
- Is any effect temporary or permanent?
- CP is heterogeneous – does aetiology or pathology affect outcome?
Who is most likely to respond?
- What are the skills required of a treatment team?
- What are the costs – financial, physical, psychological?

Prevention/early intervention in the acute phase vs treatment in the chronic phase

Stem cells safety trial

- More research (basic and rigorous trials) will be needed in the years that lie ahead
- Small safety trial using fully-matched sibling cord blood cells
 - Ethics approval received
 - Collaborative group: QLD, NSW and VIC



We remain at the crossroads...

- What is the right treatment
- For the right child
- At the right time??



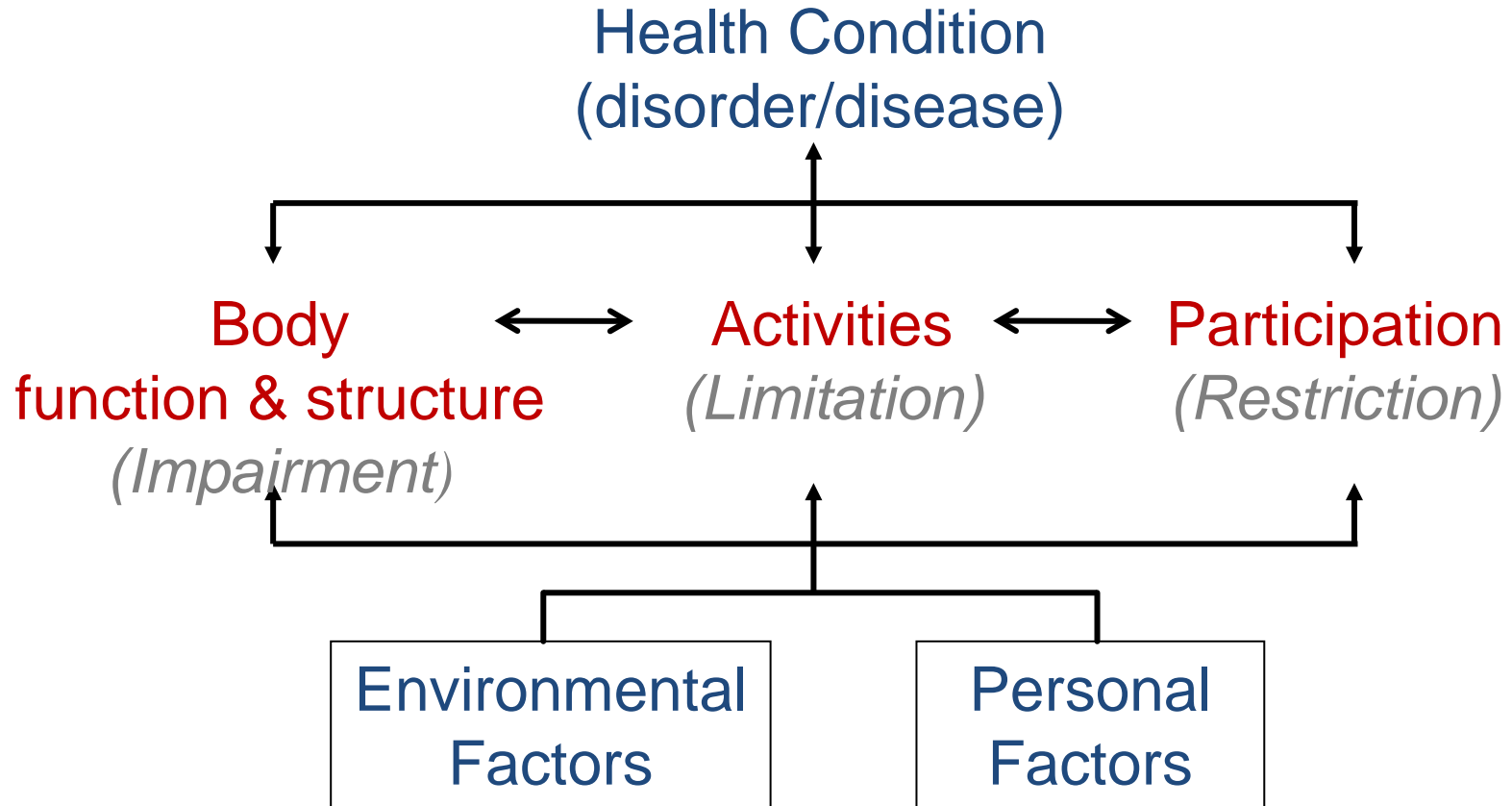
How can evidence be translated into practice and how can the research effort be increased?

But what about the child and family?

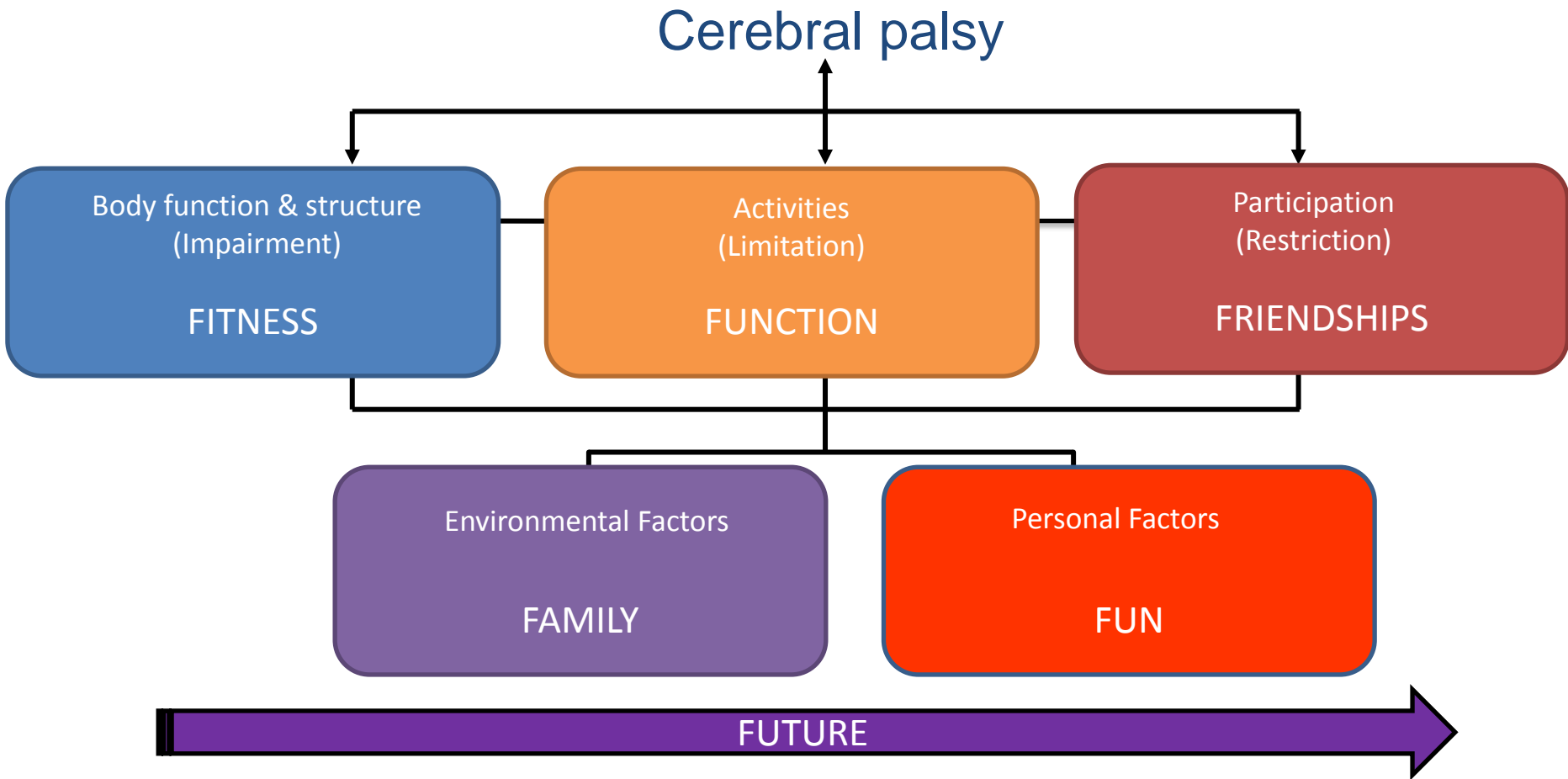
Focus shifted to:

- Promotion of function
- Value of participation
- Engagement in meaningful aspects of life

The ICF as a framework



The ICF and the F words



Fitness

- Children with disabilities are less 'fit' than other children, and less fit than they should be
- Need understanding of what makes it easy or hard for children and adolescents with disabilities to become and to stay physically active
- There is also a need for more and better recreational opportunities for all children.

Function

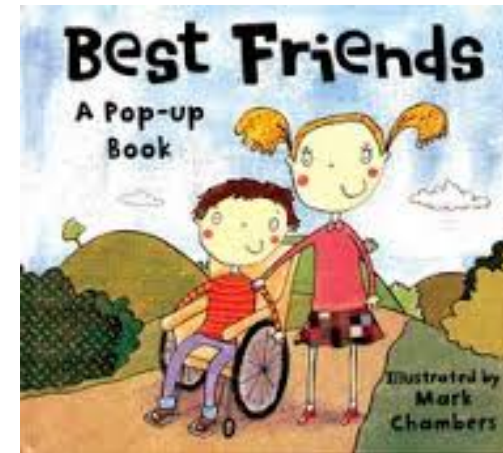
- Do not focus on “fixing” but on optimizing function
- Don’t focus on needing to learn to do things in the “normal” way – but this is not the only way that things can be done.

We need to focus on what children can do

- Open the consultation with “what has your child done well at or achieved over the past few months?”

Friends

- Social development is just as important as other aspects of learning
- Need to consider what can be done to encourage, empower and enhance children's opportunities to develop and nurture meaningful peer connections.



Family

Parents have not always been engaged

- Parents' lives are complicated with their concerns about their child
- Parental physical and mental health often challenged
- Child behaviour problems and the relationship with spouse / partner, can contribute to stress.

Think of the various supports for families, and help them to find the resources to make informed decisions.

Ask the question of parents “How are you doing? Do you need any additional help”

Fun

- Need to find out what children want to do
- Adapt activities as necessary
- Give away the expectation of expecting them to do them normally.

Future

- Need to think about the future right from the start, and encourage parents to do so as well.
- This in no way implies that we should ignore the child's and family's present realities.
- Addressing function, family, fitness, fun and friends will constantly remind us of what is important



Future

- Ask parents and children with disabilities about their expectations and dreams for a future that is possible – and not decide for them what is impossible.
- Good evidence is needed about what works and what does not work but the concepts of what is best for children and families also needs to be factored into the equation.



We remain at the crossroads

- Can we embody these concepts of fitness, function, family, fun, friendships and thinking about the future – into the work that we do??



Let's hear from an Australian with cerebral palsy: first from a young adult and then from a parent

How can we move forward to close the gaps?

We have the National Disability Insurance Scheme



NDIS | revolutionising disability services



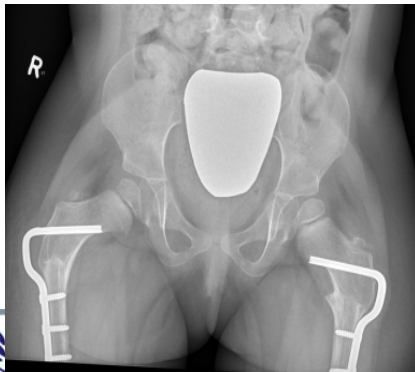
Centre of Research Excellence in Cerebral Palsy



To optimise the physical, mental, social and emotional health and wellbeing of children with cerebral palsy and their families.

Specific aims are to improve

- the functional abilities of children with cerebral palsy and
- social participation and quality of life for children with cerebral palsy and their families.



We remain at the crossroads...

- Better evidence
- More uniform care
- Improved focus on children and families

This will lead us on the pathway to a better future



FUTURE

Thank you

- Children and their families
- Tess Karambelis and Vicki Cavalieros
- CanChild – Peter Rosenbaum
- RCH Colleagues and those wider afield
- RACP
- Funders and supporters

www.cre-cp.org.au

www.rch.org.au/devmed