How do We Best Measure Broadly Defined Issues of Outcome in EOS?

James G. Wright
The Hospital for Sick Children
University of Toronto

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Objectives

- Framework for measures
- Process for choosing best measures
- Criteria to choose among measures
- Existing measures/need for measures

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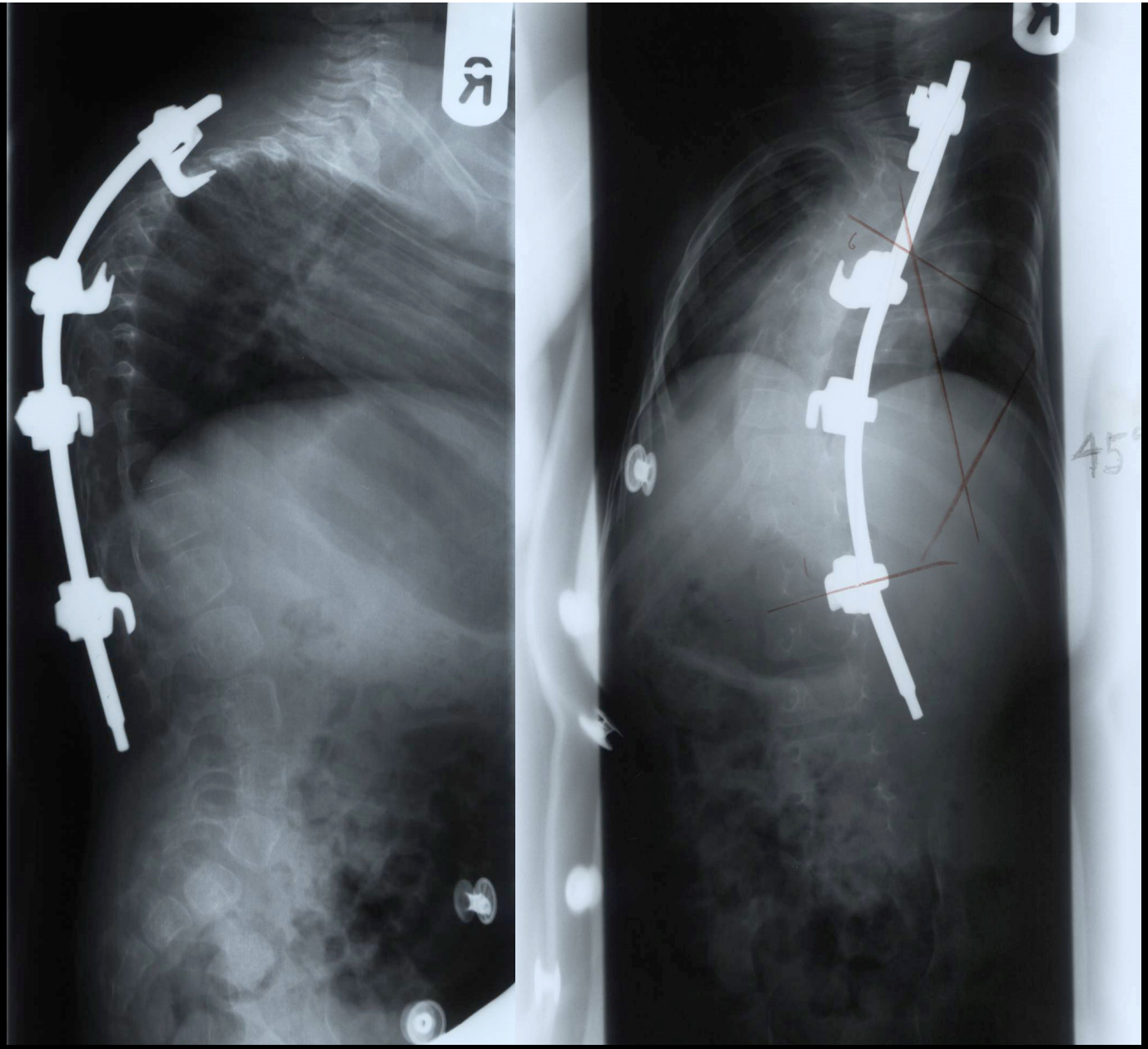
Aim of Treatment

- Treat current state: cosmesis, pain, respiratory and physical disability
- Prevent future decline: symptoms, function, death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Framework for Measures

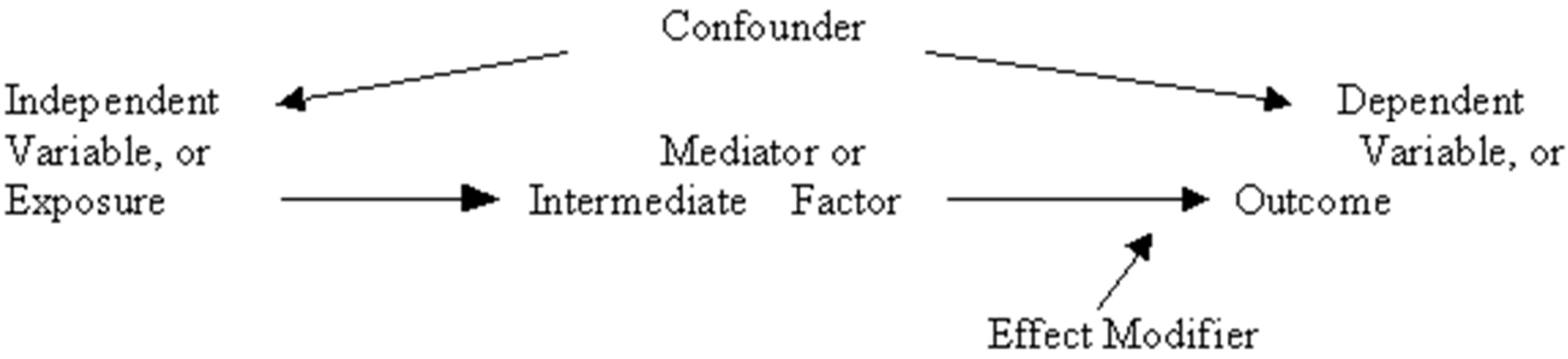
- Nonclinical: cost, length of stay
- Clinical: symptoms, signs, radiographic, laboratory/other, disease-specific health status, generic health status, quality of life, complications, death

ICIDH/ICF

The ICIDH [34,35] Compared to the Nagi Scheme [24] for the Disablement Process

ICIDH:	**Disease**	**Impairment**	**Disability**	**Handicap**
	"intrinsic pathology or disorder"	" … any loss or abnormality of psychological, physiological, or anatomic structure or function at the organ level"	*"Activity Limitation"* "… any restriction or lack of ability to perform an activity in the manner or within the range considered normal"	*"Participation"* "… a disadvantage for a given individual resulting from an impairment or disability, that limits or prevents the fulfilment of a role that is normal …"
Nagi:	**Active Pathology** "interruption or interference with normal processes, and efforts of the organism to regain normal state"	**Impairment** "anatomic, physiological, mental or emotional abnormalities or loss"	**Functional Limitations** "limitation in performance at the level of the whole organism or person"	**Disability** "limitation in performance of socially defined roles and tasks within a socio-cultural and physical environment"

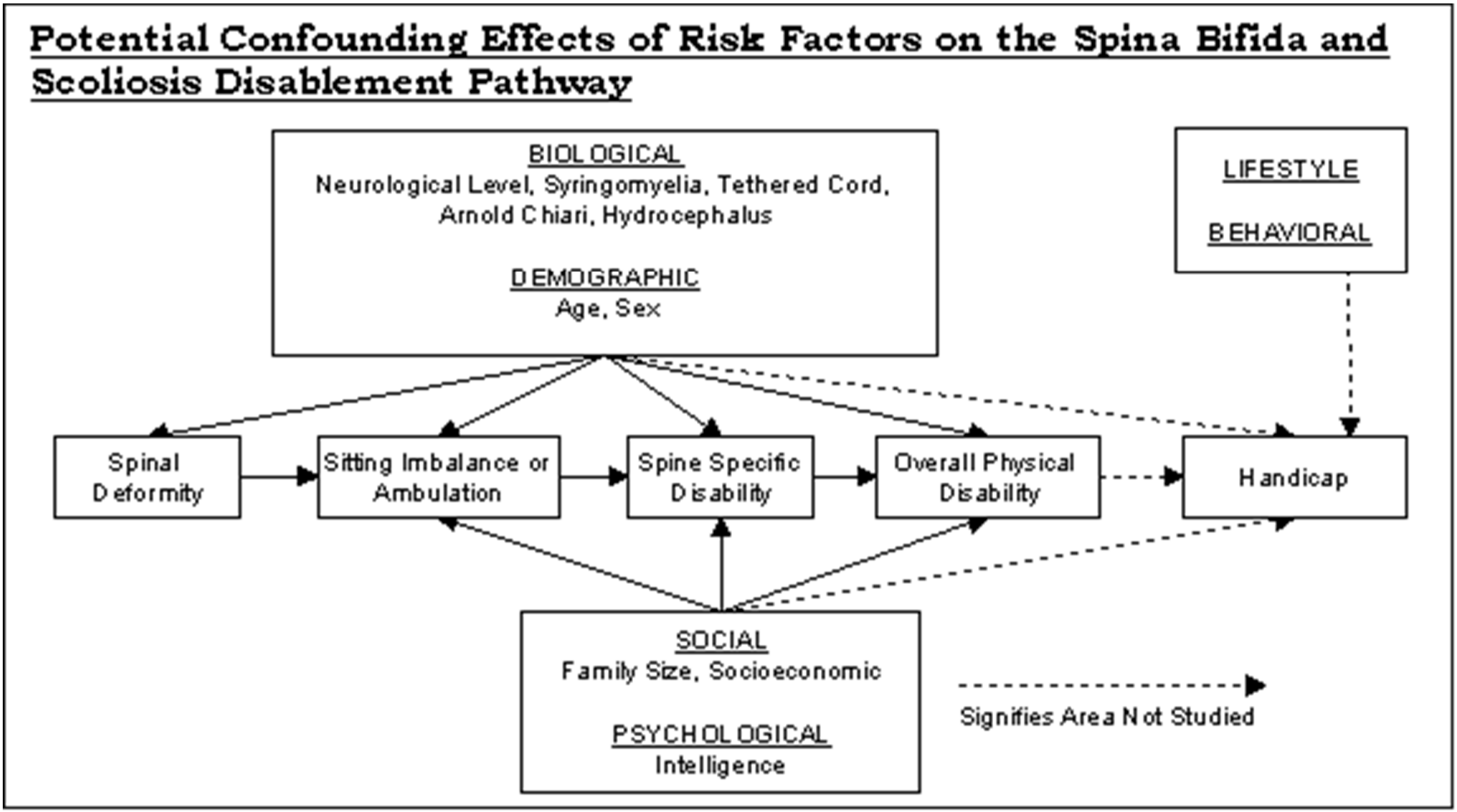
Effects of Various External Factors on the Relationship between Determinant and Outcome.



The Linear ICIDH Applied to Scoliosis in Spina Bifida

Spina Bifida →	**Spinal Deformity** →	**Physical Disability** →	**Role in Society**
(Disease)	(Impairment)	(Disability)	(Handicap)

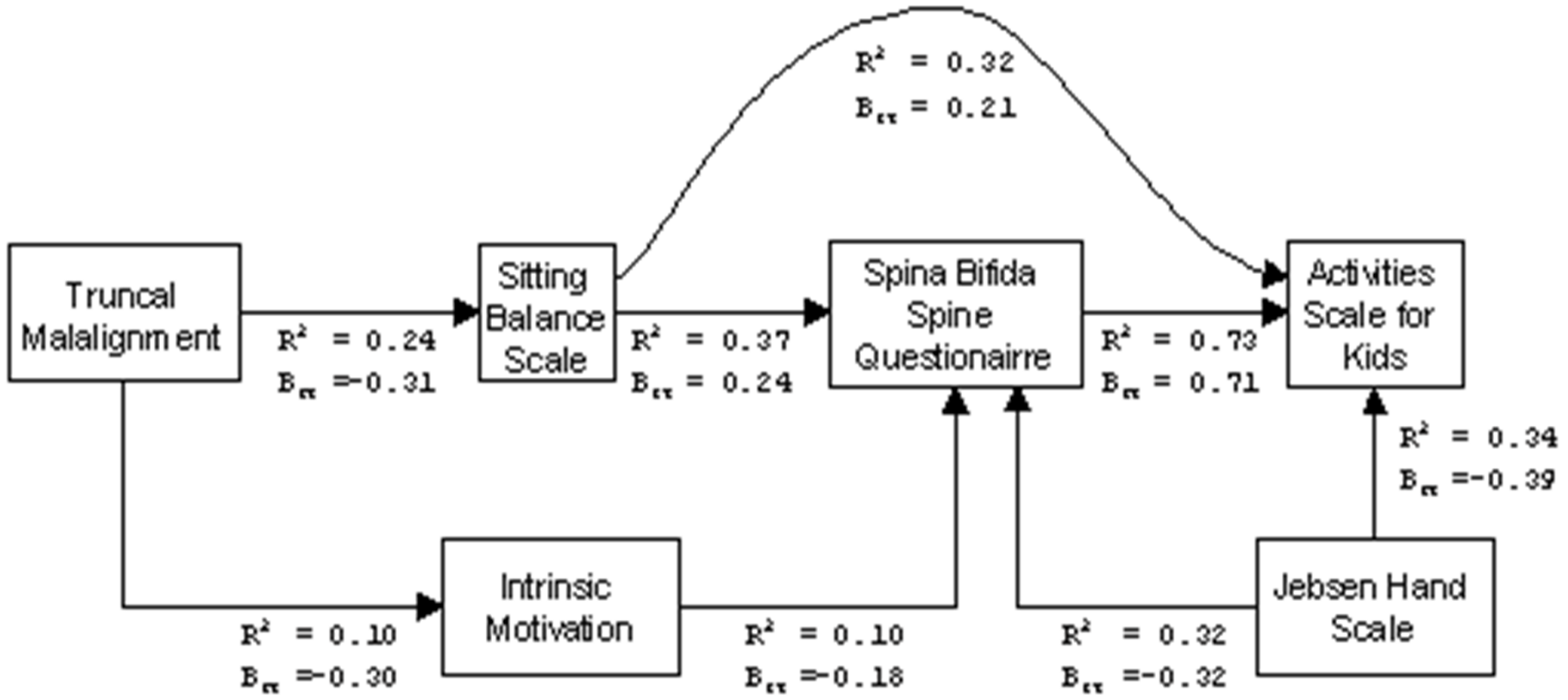
Potential Confounding Effects of Risk Factors on the Spina Bifida and Scoliosis Disablement Pathway



Summary of Relationships in the Disablement Pathway

R^2 = amount of variance explained by bivariate relationship

B_{st} = standardized beta coefficient adjusted for confounding.



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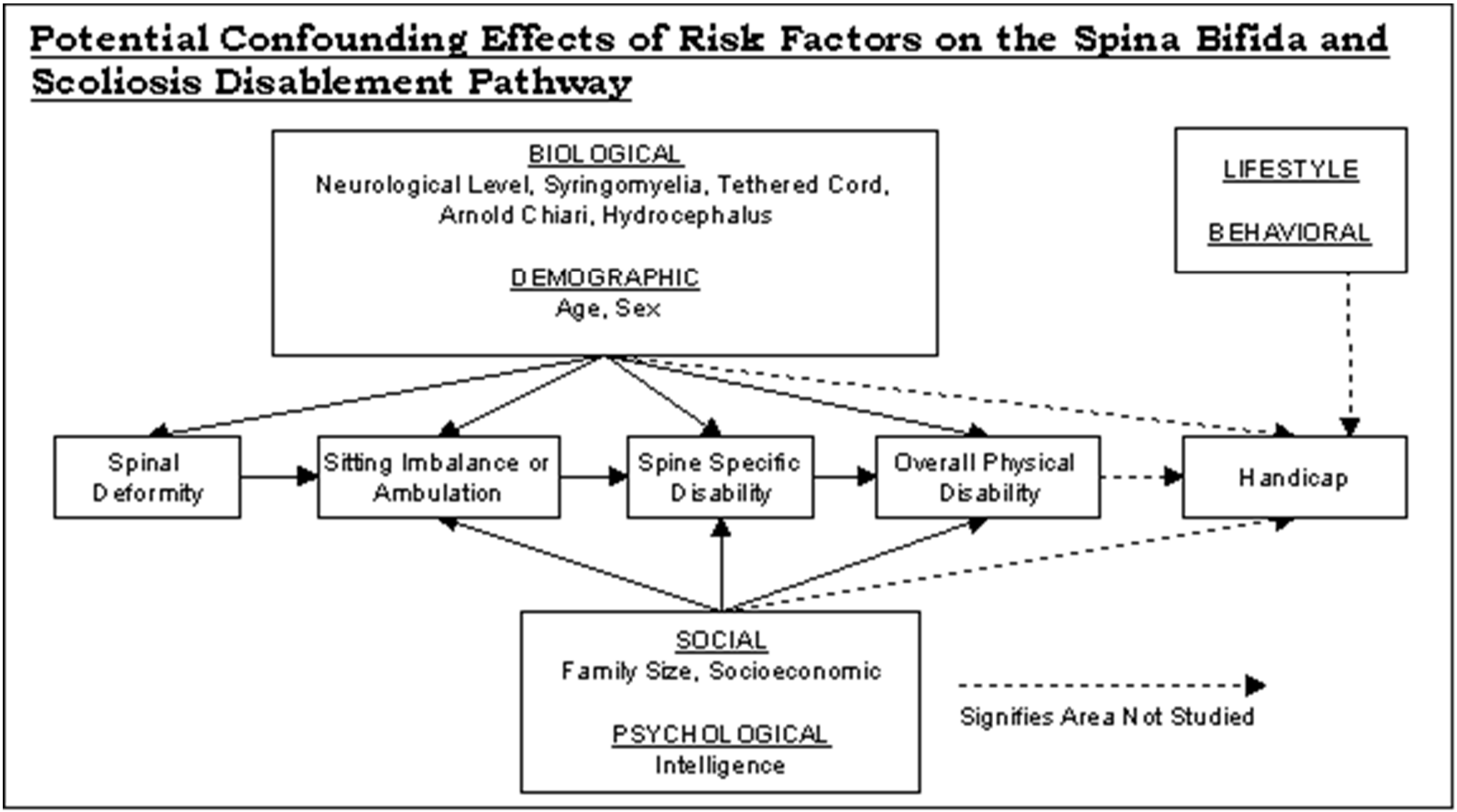
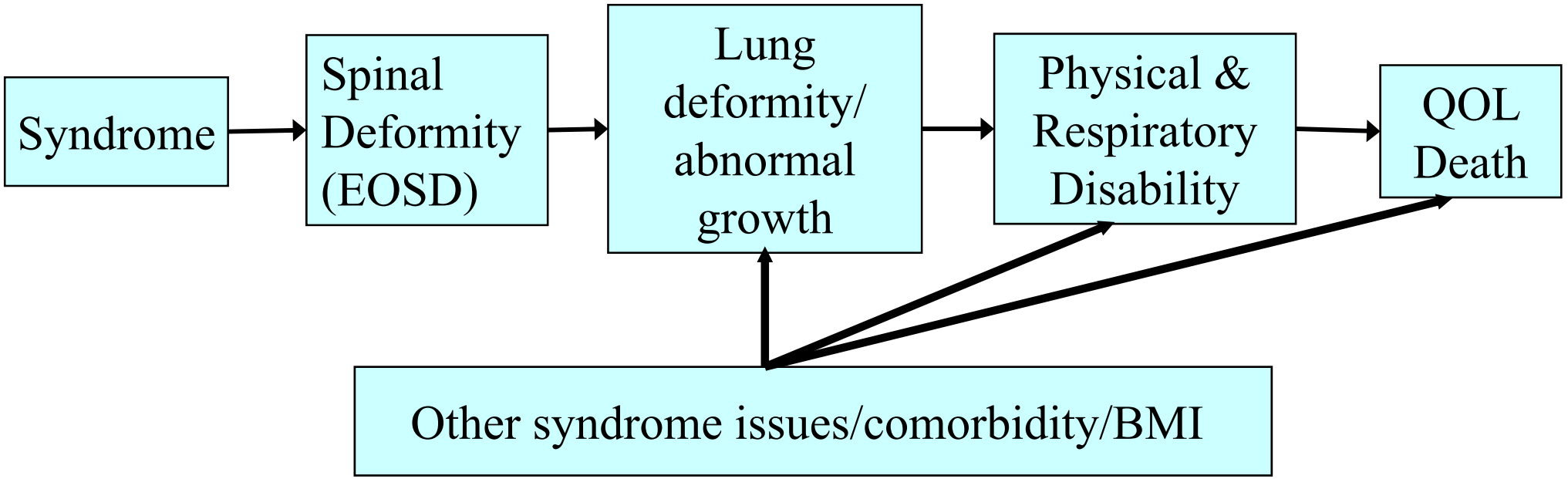



Syndrome
Spinal Deformity (EOSD)
Lung deformity/ abnormal growth
Physical & Respiratory Disability
QOL Death
Other syndrome issues/comorbidity/BMI

Potential measures

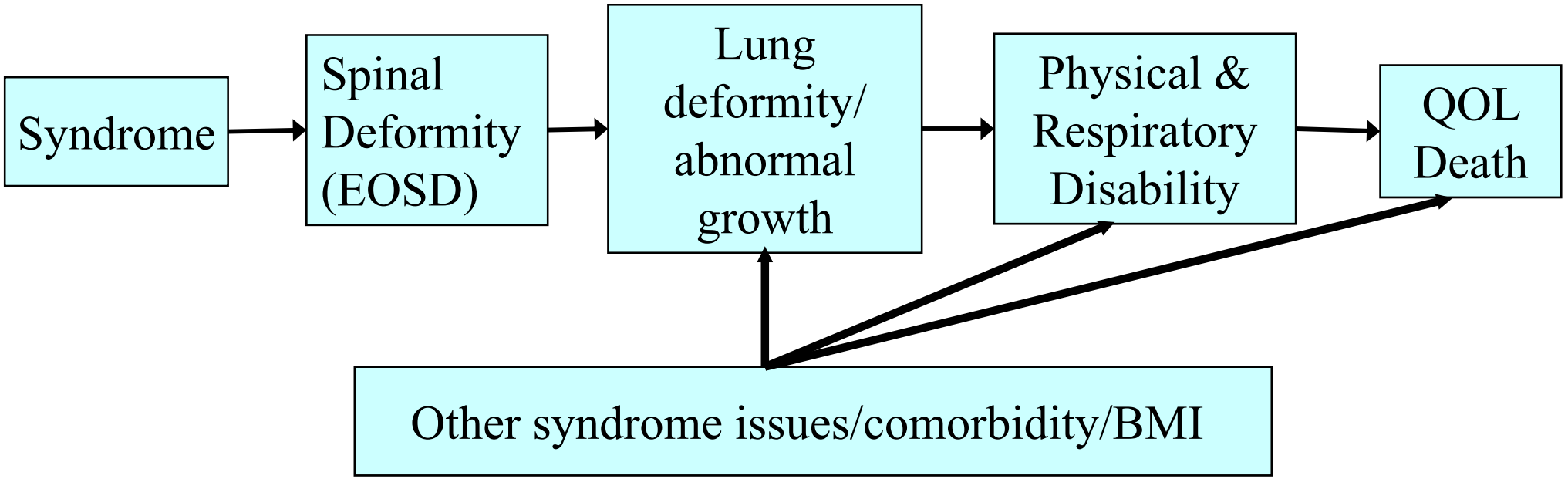
- **Anatomic:** ribs, vertebrae, muscles
- **Chest:** size, shape, compliance
- **Radiographic**: Cobb, interpedicular line ratio, decompensation, shoulder level, rotation, posterior hemithorax symmetry ratio, sagittal costophrenic depth ratio, spinal penetration index, thoracic height & depth, abdominal lung positioning, space available for lungs
- **CT**: lung area
- **PFT:** volumes, ABG/saturation, DLCO, assisted ventilation rating
- **QOL**: CHQ-PF50

Criteria for Choosing Among Measures

- Sensibility: does the measure make sense (to clinicians)
- Reliability: obtain same measure on repeated occasions
- Validity: measures what purports to measure
- Responsiveness: ability to accurately measure change

Process for Choosing Best Measure(s)

- Consensus process for minimal data set: (literature review, appropriate endorsement/participation, consensus process, dissemination, periodic revision)
- Develop needed measures: e.g. disease-specific health measure
- Examine inter-relationship of variables
- What's normal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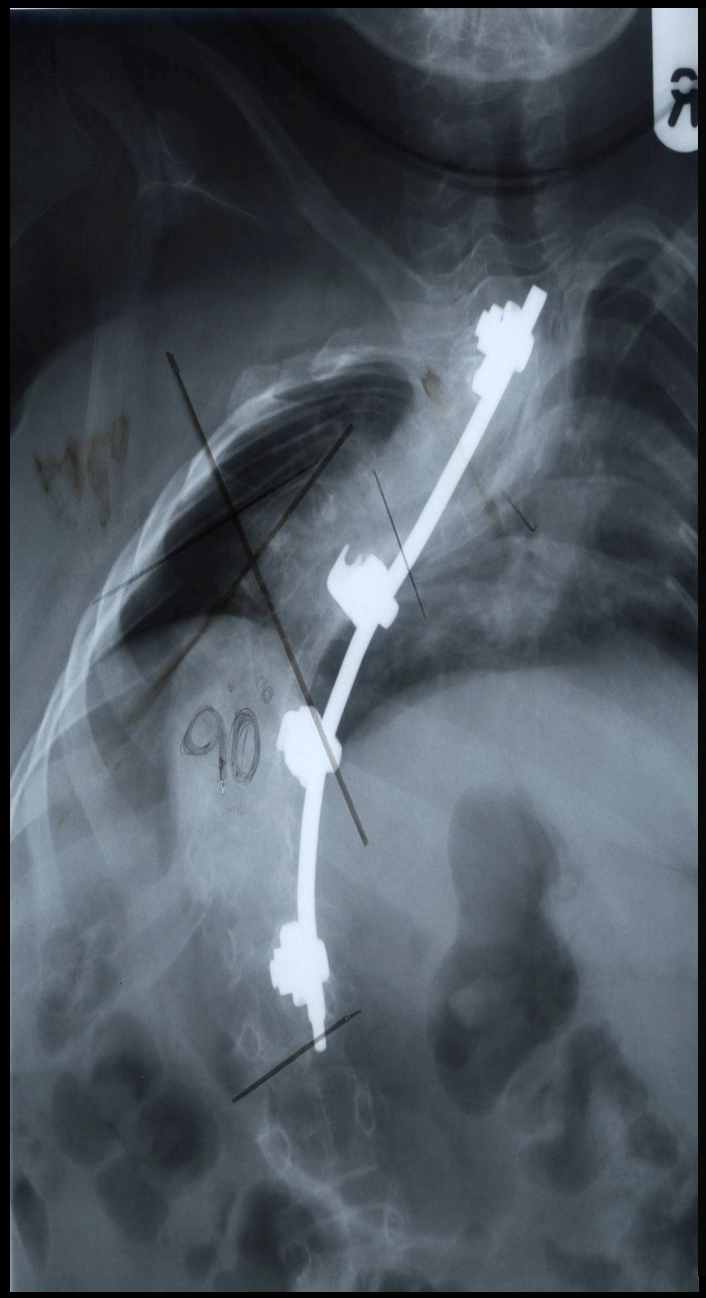
What has been published

- Early fusion associated with worse PFTs (Goldberg 2003)
- CT lung volumes related to PFTs; shorter, higher, more rotated curves associated with lower CT volumes, worse R>L (Adam 2007)
- More extensive fusions and more proximal fusions associated with worse PFTs (Karol 2008)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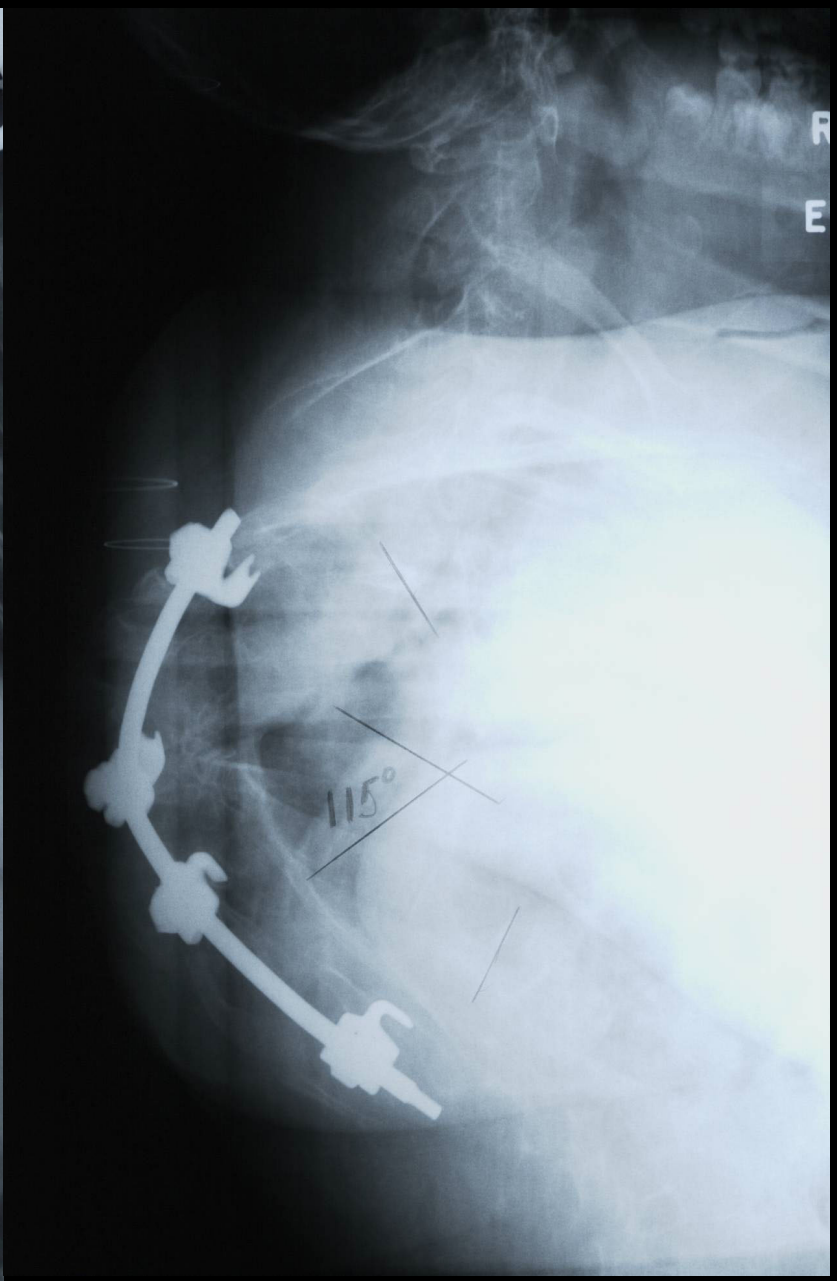
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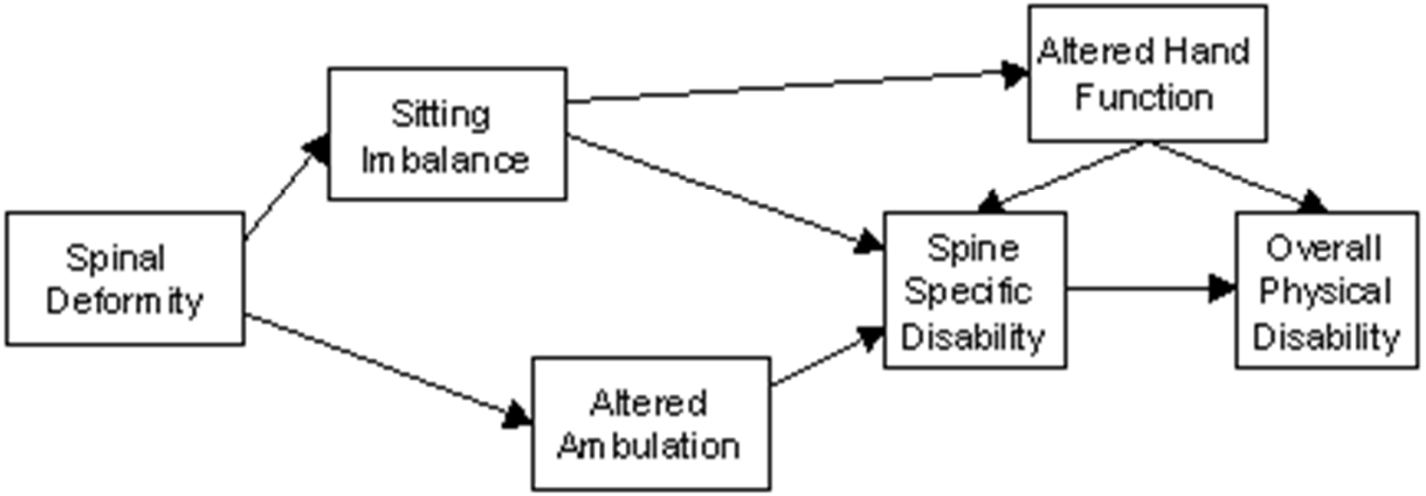
Conclusion

- Develop a minimal data set (by consensus)
- Develop a disease-specific measure
- Evaluate inter-relationship of measures
- Evaluate change with time or treatment

Hypothesized Primary Intermediate Steps in the Relationship Between Spinal Deformity and Overall Physical Disability



Verbrugge Classification of External Factors in the Disablement Process [31]

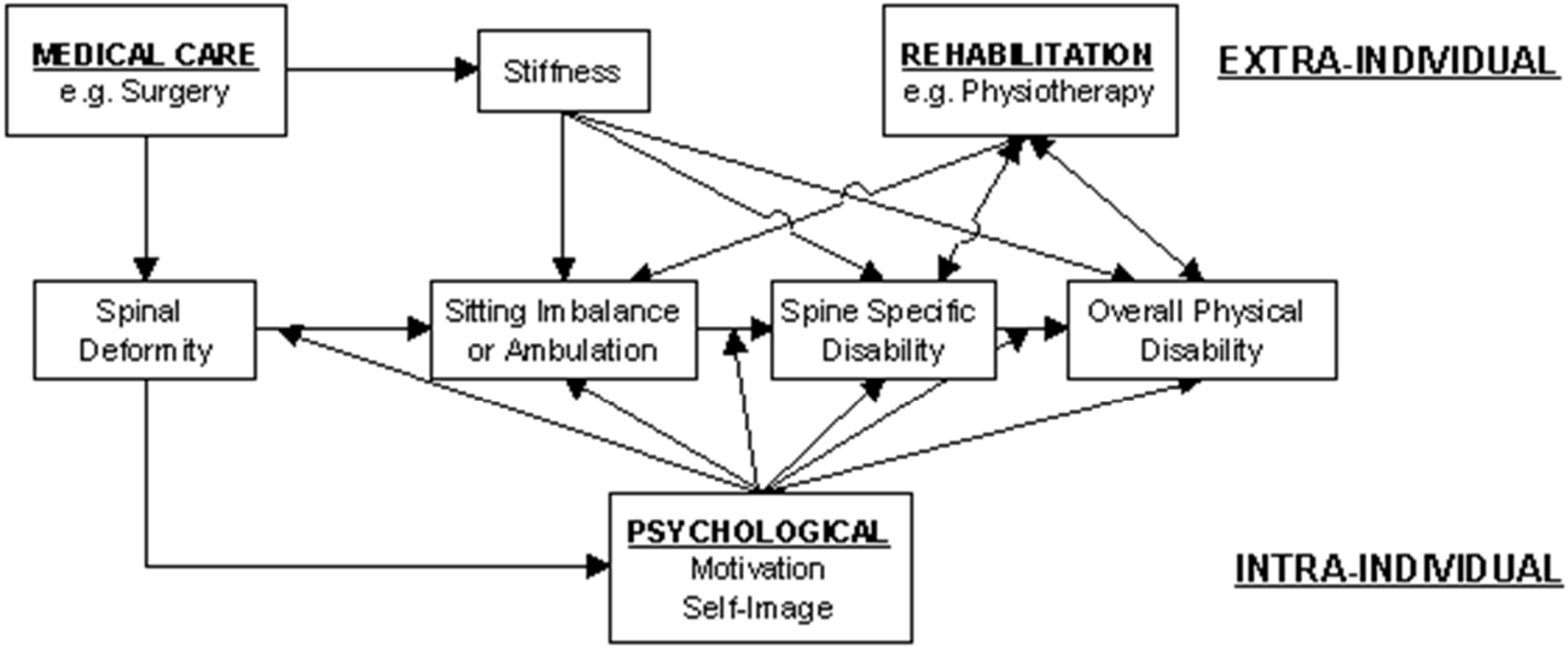
Risk Factors
(Pre-existing characteristics)

- Biolgocial
- Demographic
- Social
- Behavioral / Lifestyle
- Psychological

Intra-Individual Interventions

- Lifestyle and Behavior
  (Overt changes to alter disease activity and impact)
- Psychosocial Attributes
  (Positive affect, emotional vigor, prayer, locus of control, cognitive adaptation to one's situation, peer support group etc.)
- Activity Accommodations
  (Changes in kinds of activities, procedures for doing them, length of time doing them etc.)

Extra-individual Interventions

- Medical care and rehabilitation
  (Surgery, physical therapy, speech therapy, counseling, health education, job retraining, etc.)
- Medication and other therapeutic regimens
  (Drugs, recreational therapy/aquatic exercise, biofeedback/meditation, energy conservation, etc.)
- External supports
  (Personal assistance, special equipment, day care, respite care, meals-on-wheels etc.)
- Social and physical environment
  (Employment discrimination, accesses to buildings and public transportation, structural modifications at home/job, health insurance and access to medical care, income supports, laws and regulations, etc.)

Interventions and their Potential Effects on the Spina Bifida and Scoliosis Disablement Pathway