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What are the best nutritional outcome measures?

Helen White

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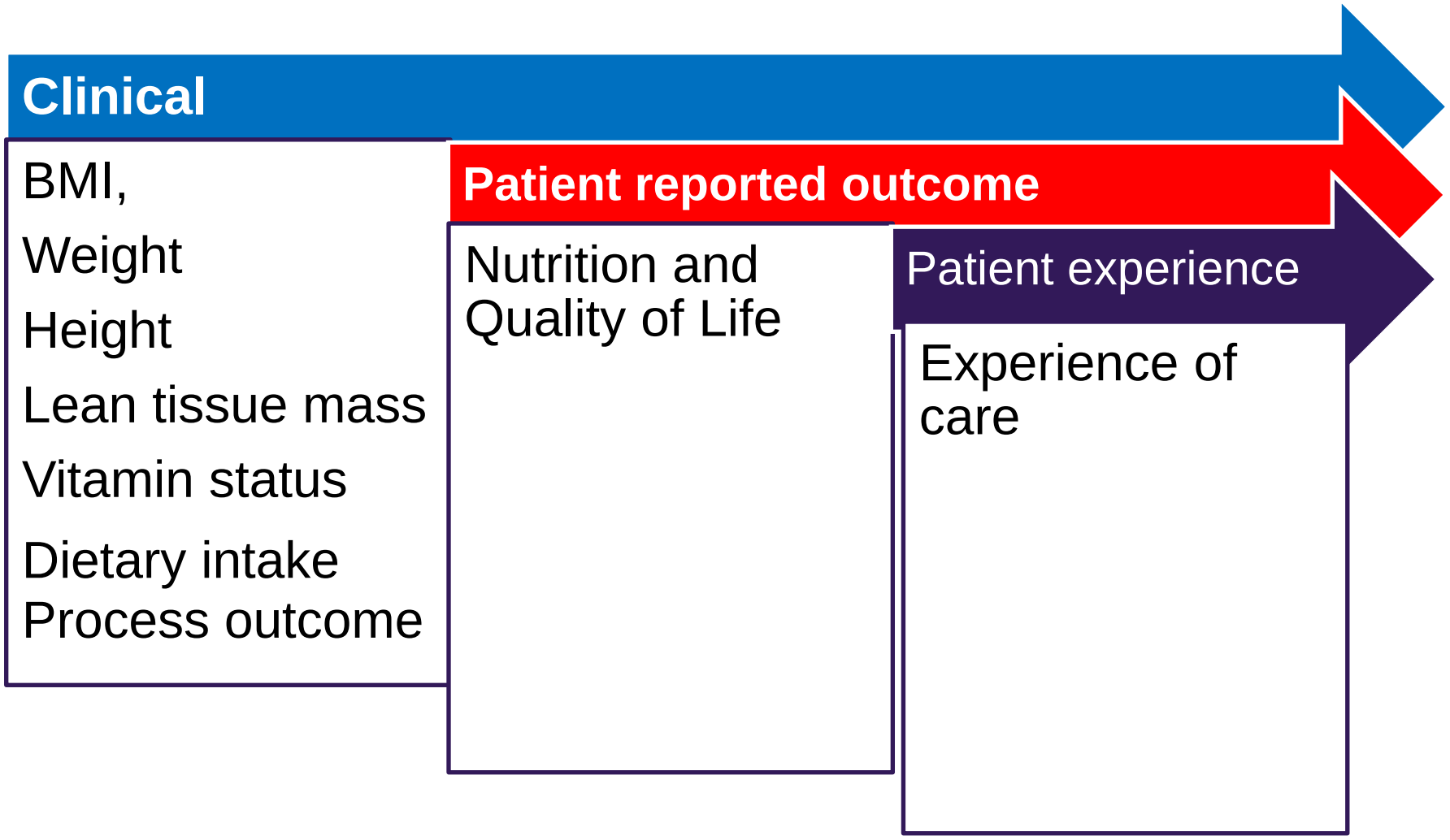
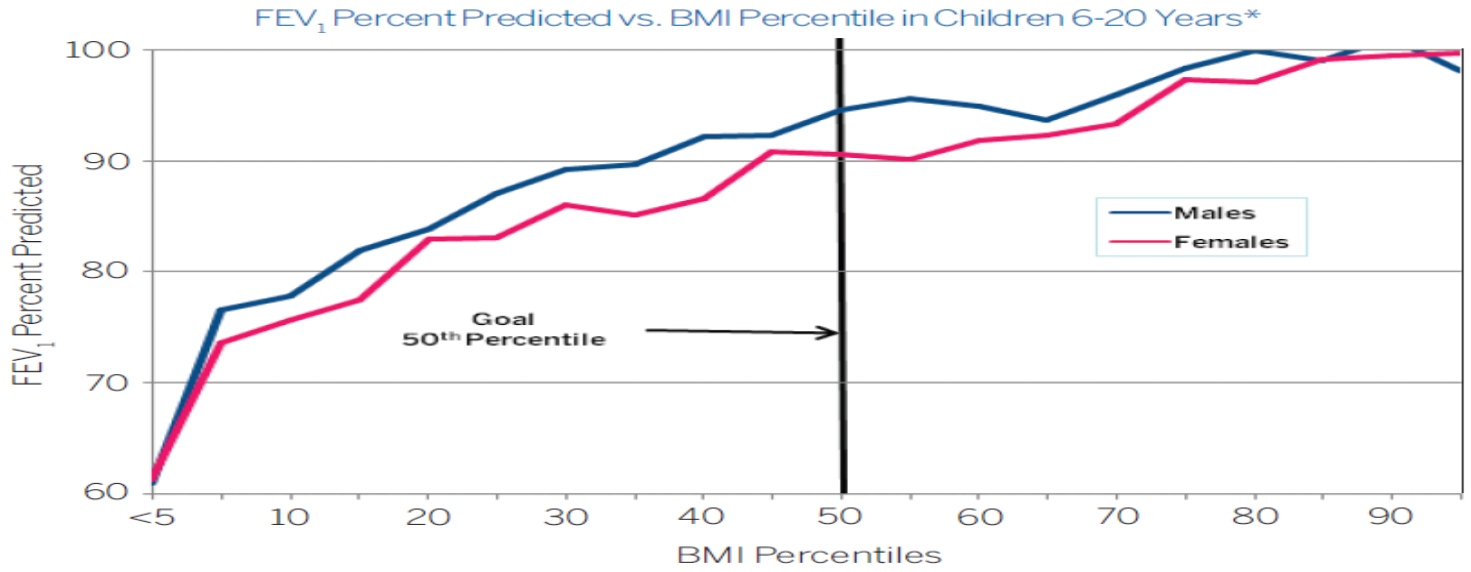
What is the value of nutritional outcome measurement?

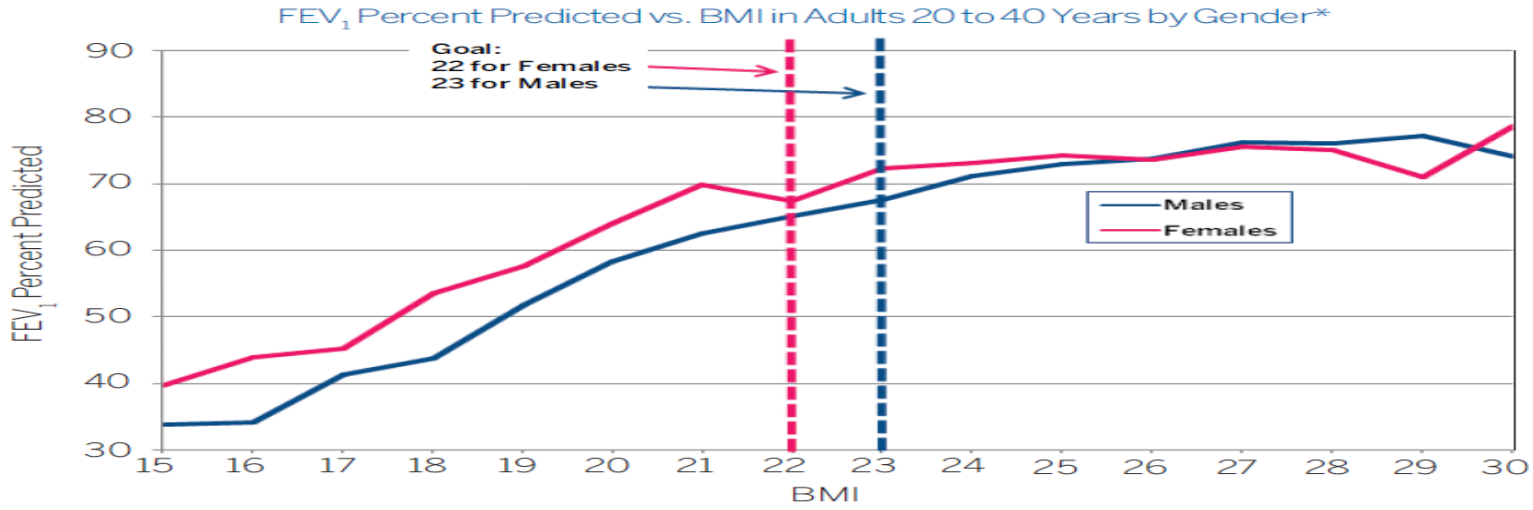
Clinical use of data	Benchmarking and quality improvement
Quality measure in contracts	Pay for performance
Patient choice	Choosing high quality providers; informed treatment choices
Resource allocation; productivity measures	Allocate scarce resources more efficiently
Tackling Health Inequalities	Appropriate access for needs
Regulation	Assessing minimum standards
National accounting	Outcomes Frameworks and productivity

Nutritional outcome measures



Established clinical measures - BMI





Advantages of reporting by BMI percentile and BMI

- Evidence based 50th percentile cut-off value based on its association with lung function
- Directly derived from data from the general population
- Superior to Ht/age and Wt/age
- More sensitive to changes in stature and age
- More sensitive to changes in FEV1 than %IBW and had a stronger association to FEV1 than %IBW
- Directly interchangeable with z-scores
- Forms a practical and sensitive measure for comparison and benchmarking

- Weidemann et al 2007 German registry data confirmed
  - BMI as a more sensitive outcome measure
  - Close correlation of BMI percentile with wt/age percentile but not ht/age

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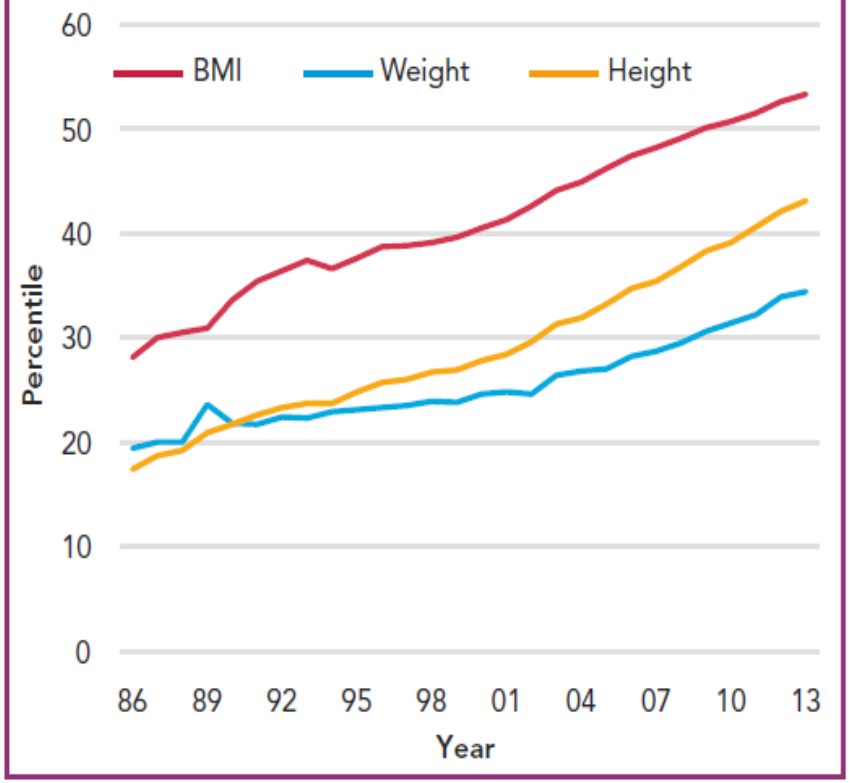
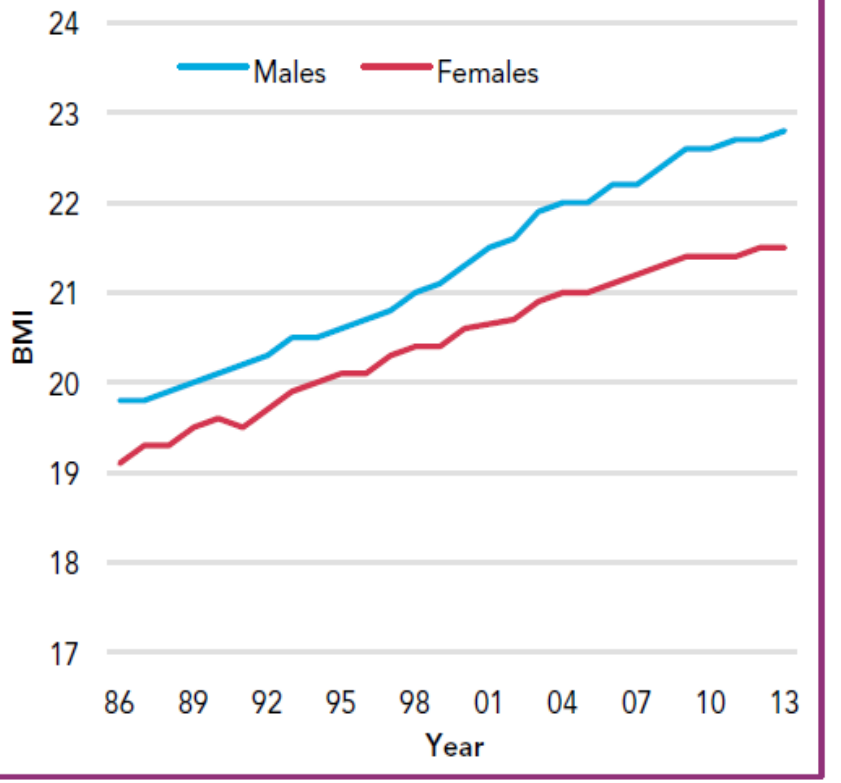
Longitudinal comparisons

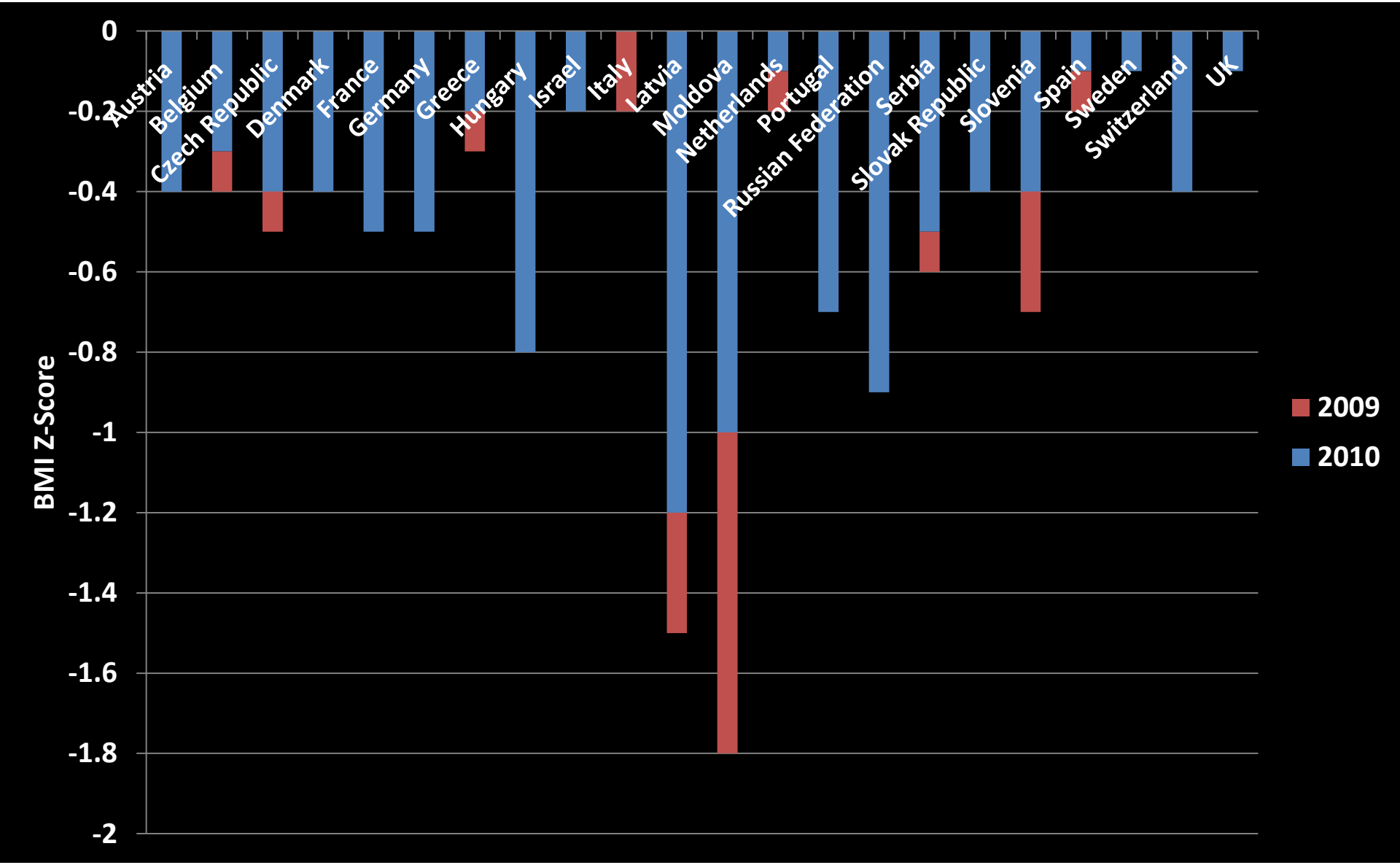
Median Nutritional Outcome Percentiles for Patients 2 to 19 Years, 1986–2013



Median BMI Value for Patients 20 Years and Older, 1986–2013



BMI Z-Scores (European CF Registry 2009 v 2010)



BMI percentile - disadvantages

	Boys 2-18 years old		Girls 2-18 years old	
BMI Percentile	**WFA <10%**	**HFA <10%**	**WFA <10%**	**HFA <10%**
≥ 50	3.5%	21.6%	2.7%	19.3%
25 < 50	16.0%	27.2%	16.7%	25.9%
< 25	39.3%	31.3%	38.2%	29.7%

Konstan et al., 2014

- 1/5 of children with a BMI above the 50th centile had a height for age below the 10th centile
- As did 1/4 with BMI between the 25th and 50th centile

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Argument against use of BMI as a single outcome measure

- Changes in US - 1/3 centres changed by more than a tertile in rankings
- 2 of the best 5 centres on nutritional failure moved to the bottom tertile for below BMI goal
- Identified that using a single outcome measure can be misleading and that BMI goal outcome measure should not be the sole basis for making treatment decisions and identifying the best centres against which to benchmark best practice.
- ***Other indicators*** such as abnormal status of fat soluble vitamins should also be used
- Careful monitoring of the ***processes of care*** are also required

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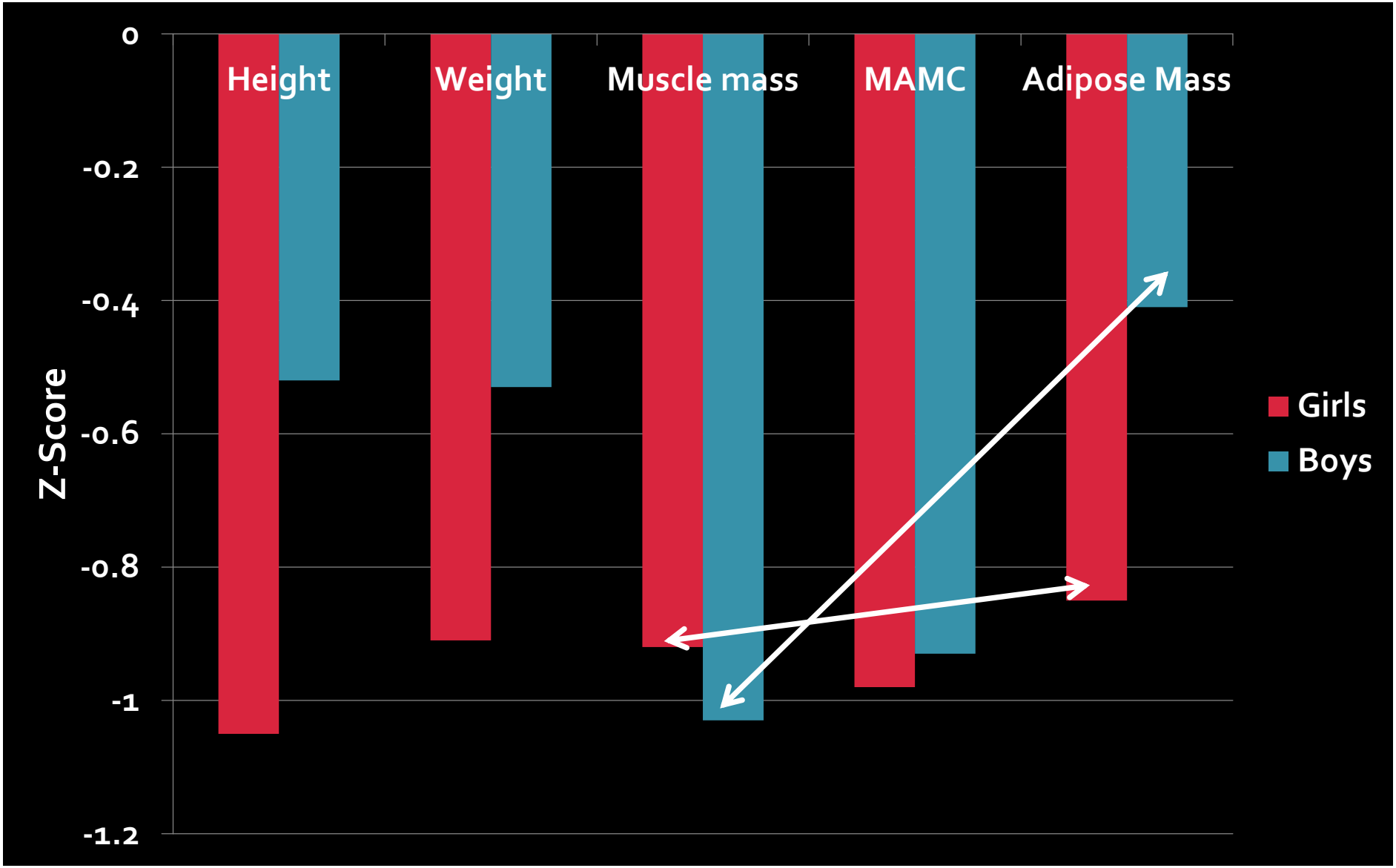
Stapleton et al, 2001



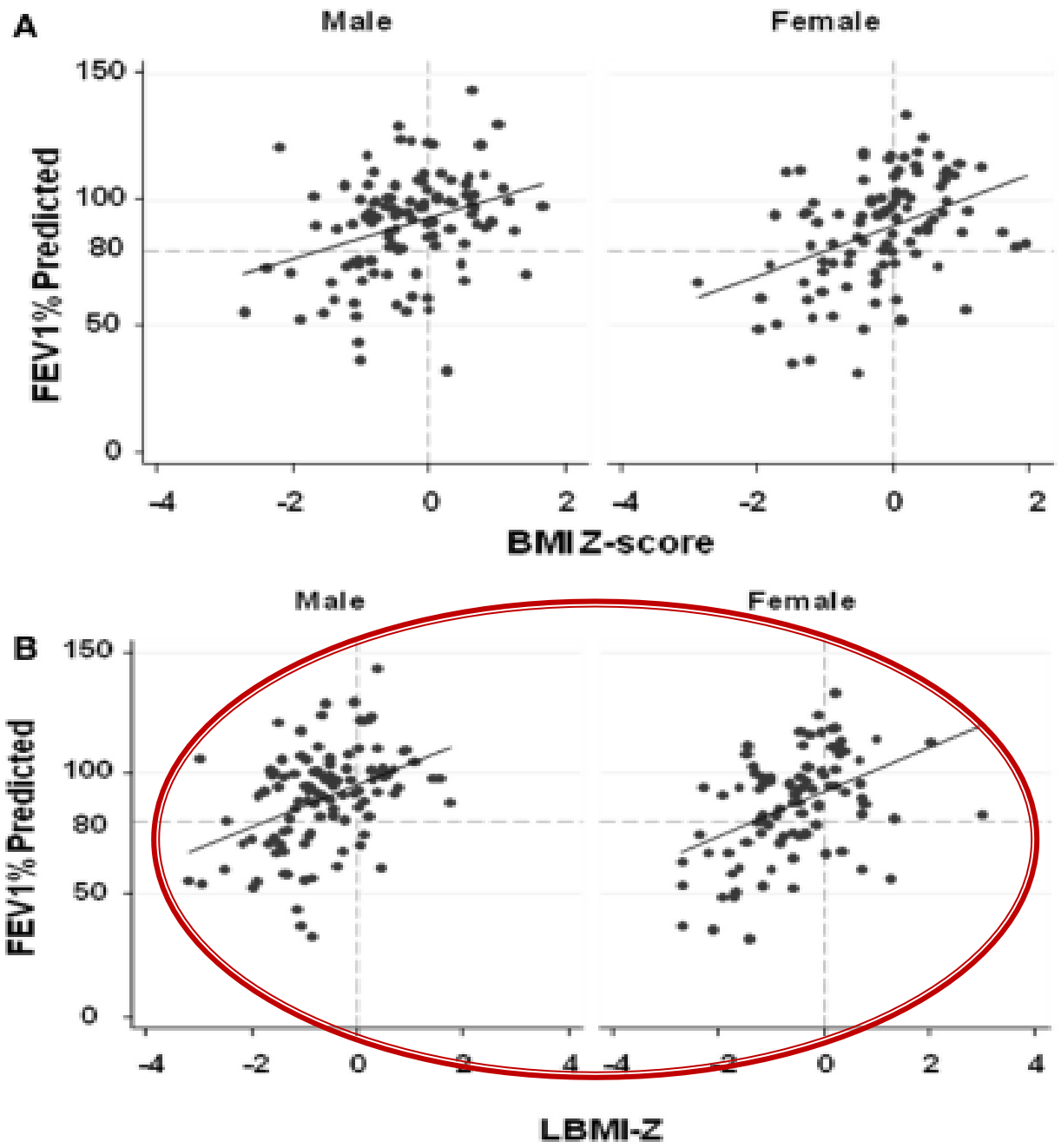
Depletion of fat free mass

Studies	Age	Fat free mass depletion	BMI depletion	Association
Ahmed et al, 2004 (FFM)	10-16 yrs	7.6% girls 10.7 % boys		FEV1 (boys only)
Reix et al, 2010 (LTM)	<6 yrs <10 yrs	12% 10%		Clinical status
Williams et al, 2010 (FFM)	6-12 yrs			Not associated with FEV1
King et al, 2010 (FFM)	Adults	14%	58%	FEV1
Ionescu et al, 2003 (FFM)	Adults	57%	40%	Disease severity Reduced BMD Systemic inflammation
Fogarty et al, 2011 (LBM)	Adults		-	FEV1
Rochat et al, 1994 (LBM)	Adults	66%	-	BMD
Hollander et al, 2014 (FFM)	Adults	60%	39%	Survival post Tx

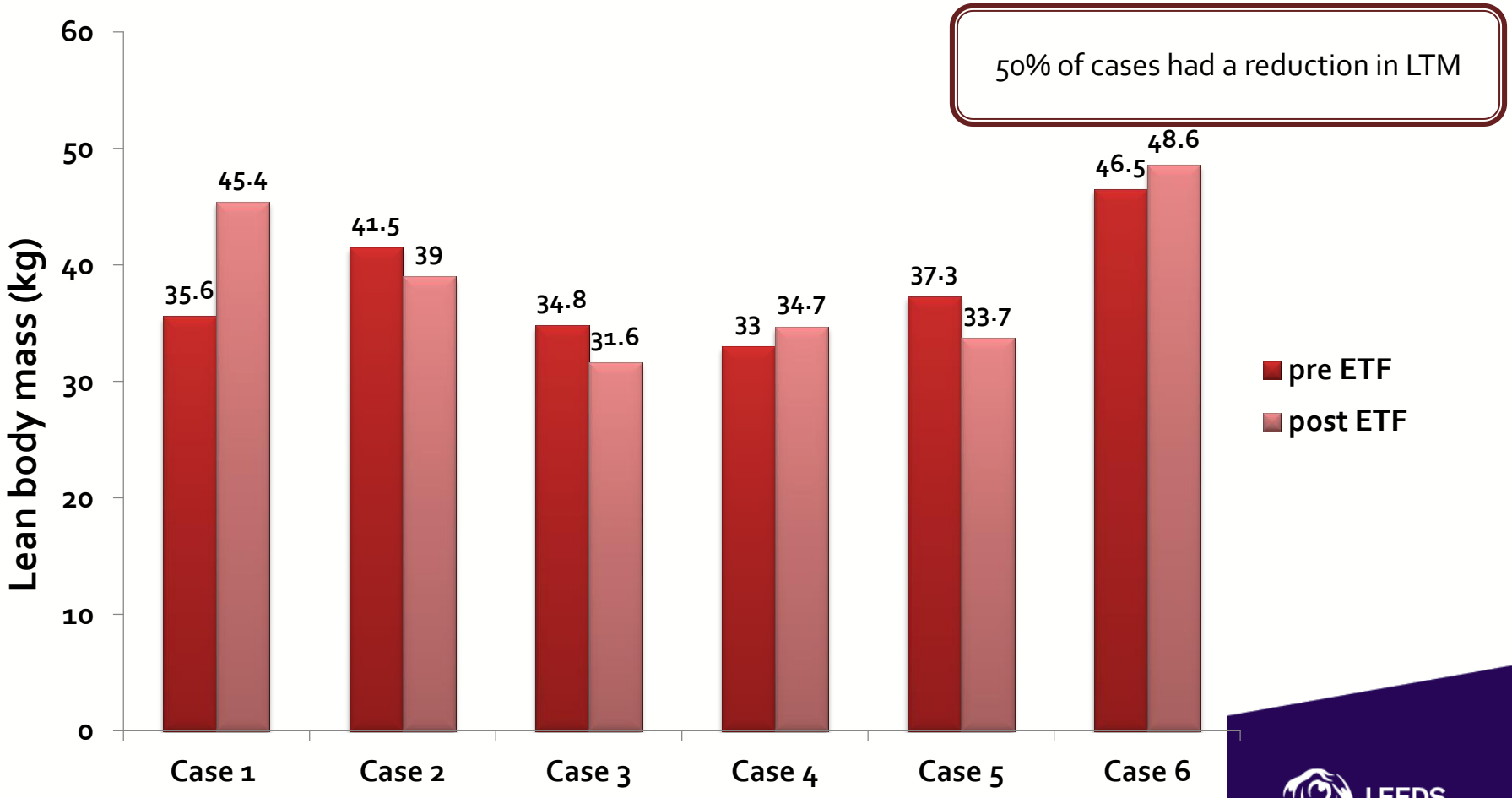
Sheikh et al, 2014

High likelihood of normal lung function, targeting BMI z-score > 0

Improved further, by targeting LBM z-score >0



Changes in lean tissue mass from pre to post-start enteral tube feeding



White et al., 2014

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How we assess lean tissue mass and fat free mass

- Surrogate measures for nutritional outcomes for LTM
- Handgrip strength
- BIA
- DEXA

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Nutritional outcomes and vitamin and mineral status

- Calcium
- Vitamin D
- Vitamin A
- Vitamin K

- All have target levels and norms specified by current guidance

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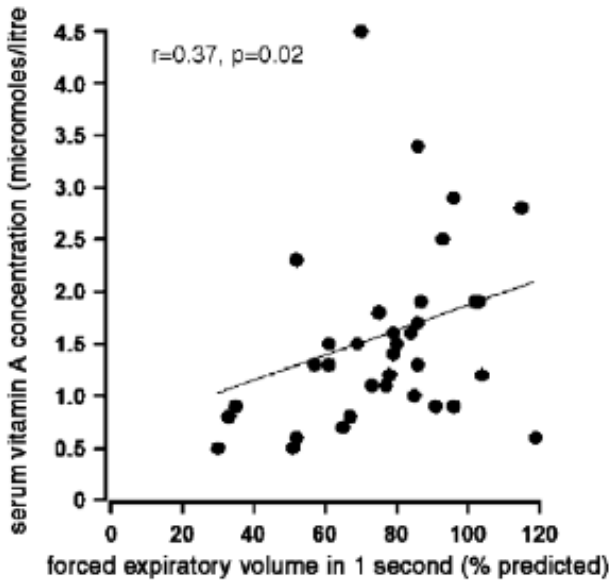
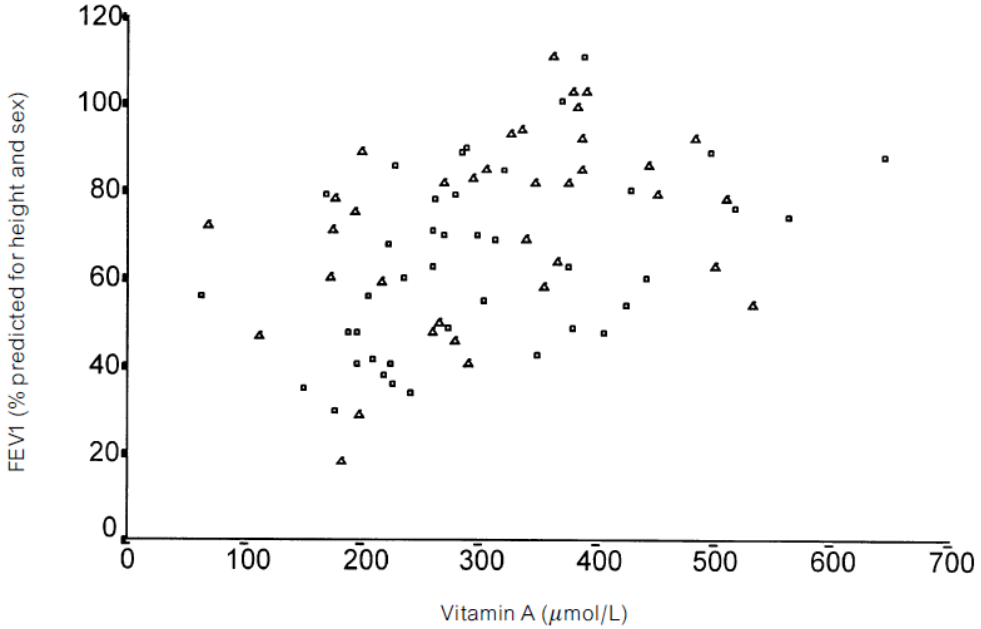
Vitamin A and outcome



Aird et al. 2006



Carr et al. 1996

Vitamin A associated with CRP inflammation (Greer et al, 2003), pulmonary exacerbation (Duggan et al, 1996; LaGrange et al, 2004

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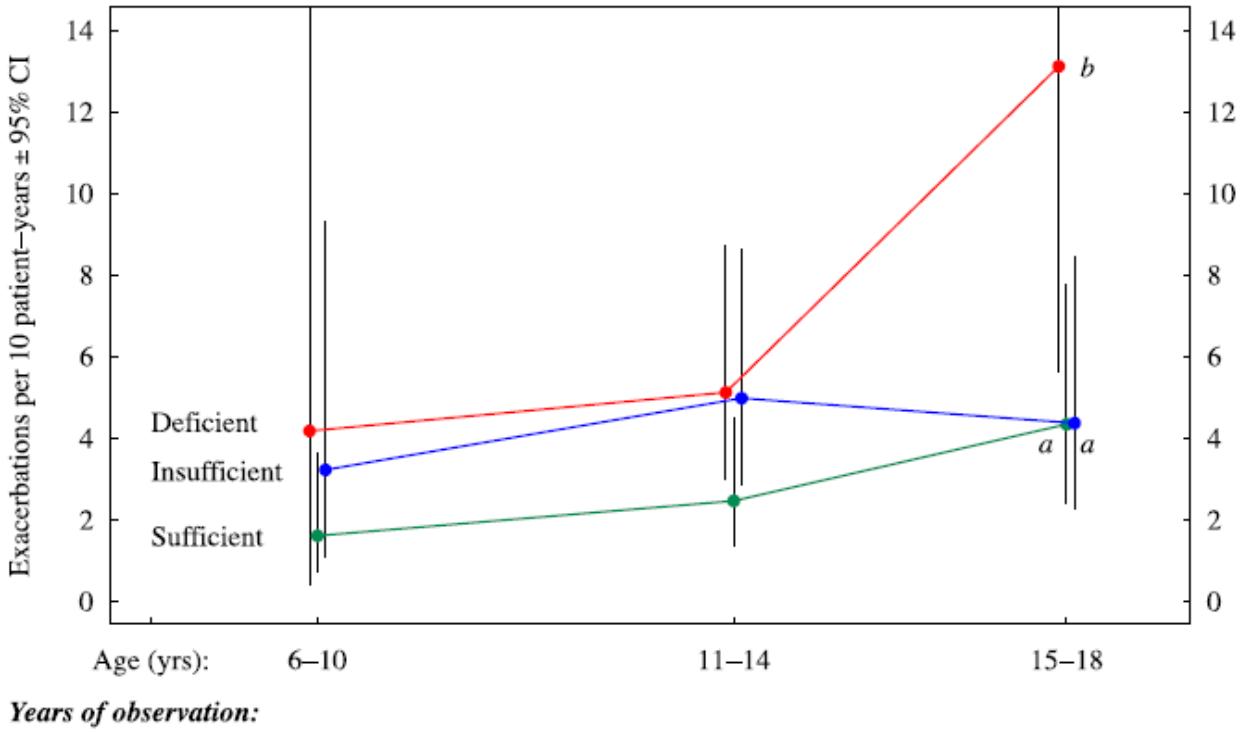
Vitamin D and outcome



McAuley et al, 2014

Most studies have shown no association between Vitamin D and lung function

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Nutritional outcomes – and dietary recommendations

- Achievement of ***minimum*** of 110% EAR in pancreatic insufficiency
- Studies that have examined energy intakes before and after interventions have indicated consistent improvements in:-
  - Weight
  - Wt/age
  - %IBW
  - BMI z-score
  - BMI

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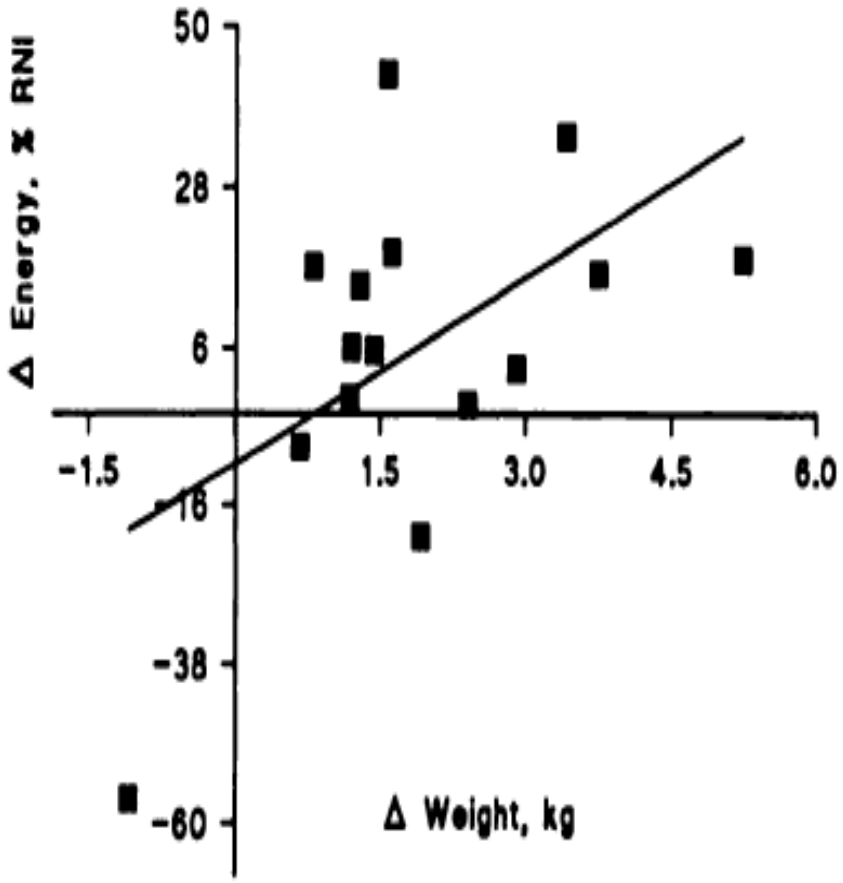
Hanning et al 1993



- Energy intake improved from 103% EAR to 122 % EAR.
- Positively correlated with weight gain

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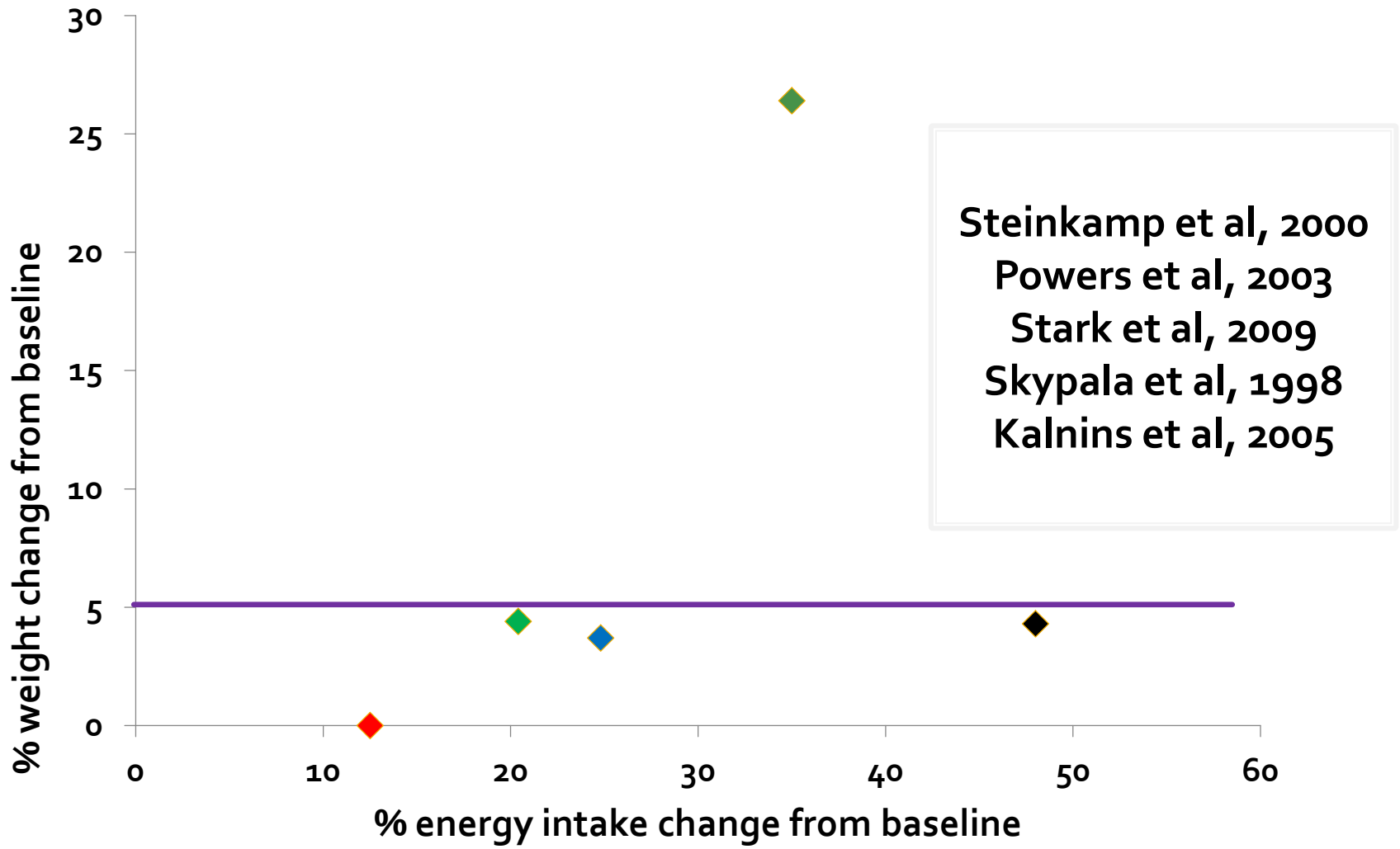
% energy intake change versus % weight change with nutritional interventions



Calcium intake targets

Age	Intake
0-6 months	210mg
7-12 months	270mg
1-3 years	500mg
4-8 years	800mg
9-18 years	1300mg
19-50years	1000mg
>50 yrs	1200mg

Sermet-Gaudelus et al, 2011

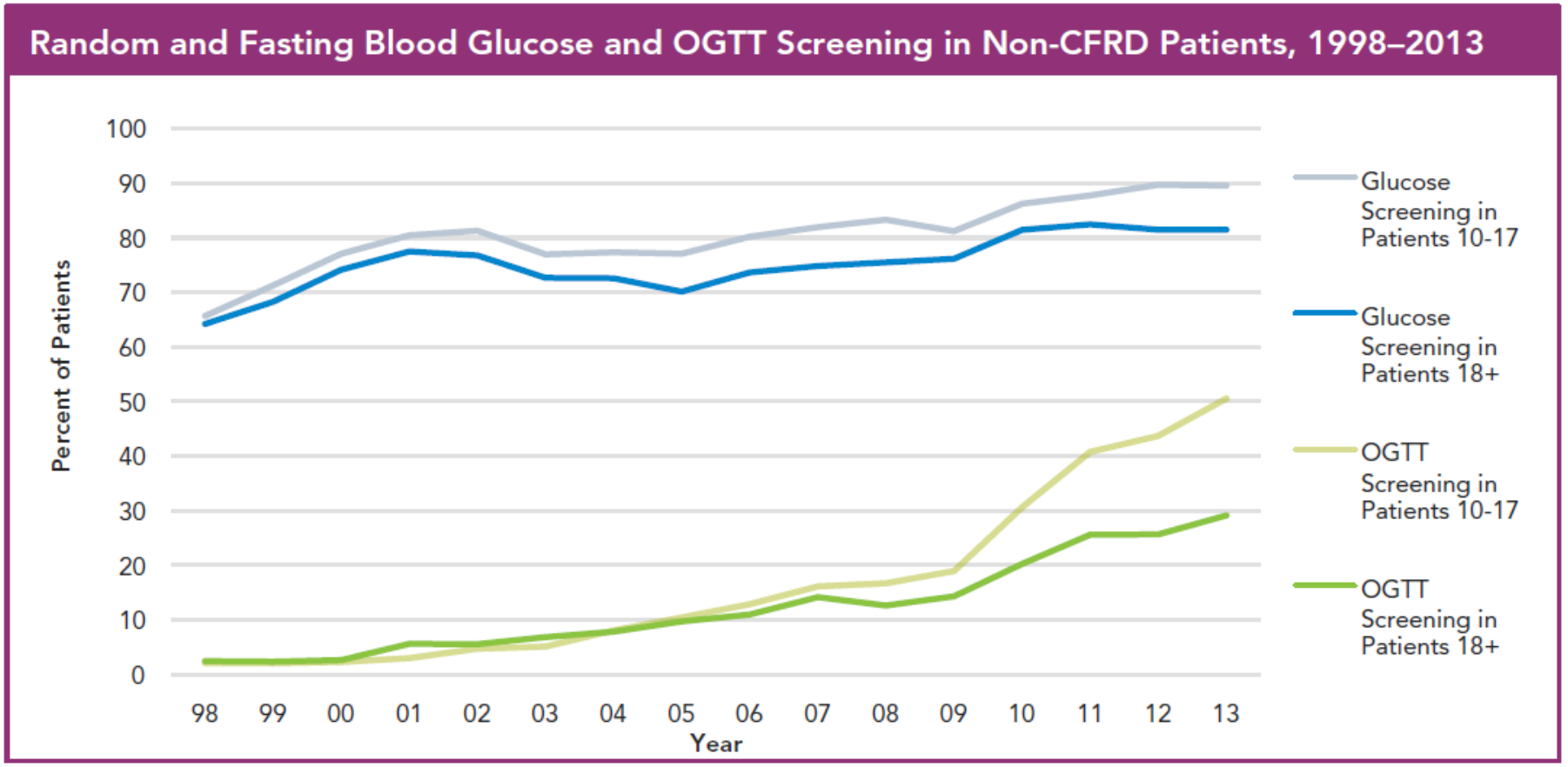
Calcium absorption

	Population	Pre puberty	Early puberty	Late puberty
Abrams & Stuff (1994	Healthy children	27.7%	34.4%	25%
Shulze et al, 2003	Children with CF (clinically stable, well nourished)	26.7%	39.9%	29.8%

Suggests that absorption is comparable to healthy children

Lower gut pH in CF may enhance calcium absorption

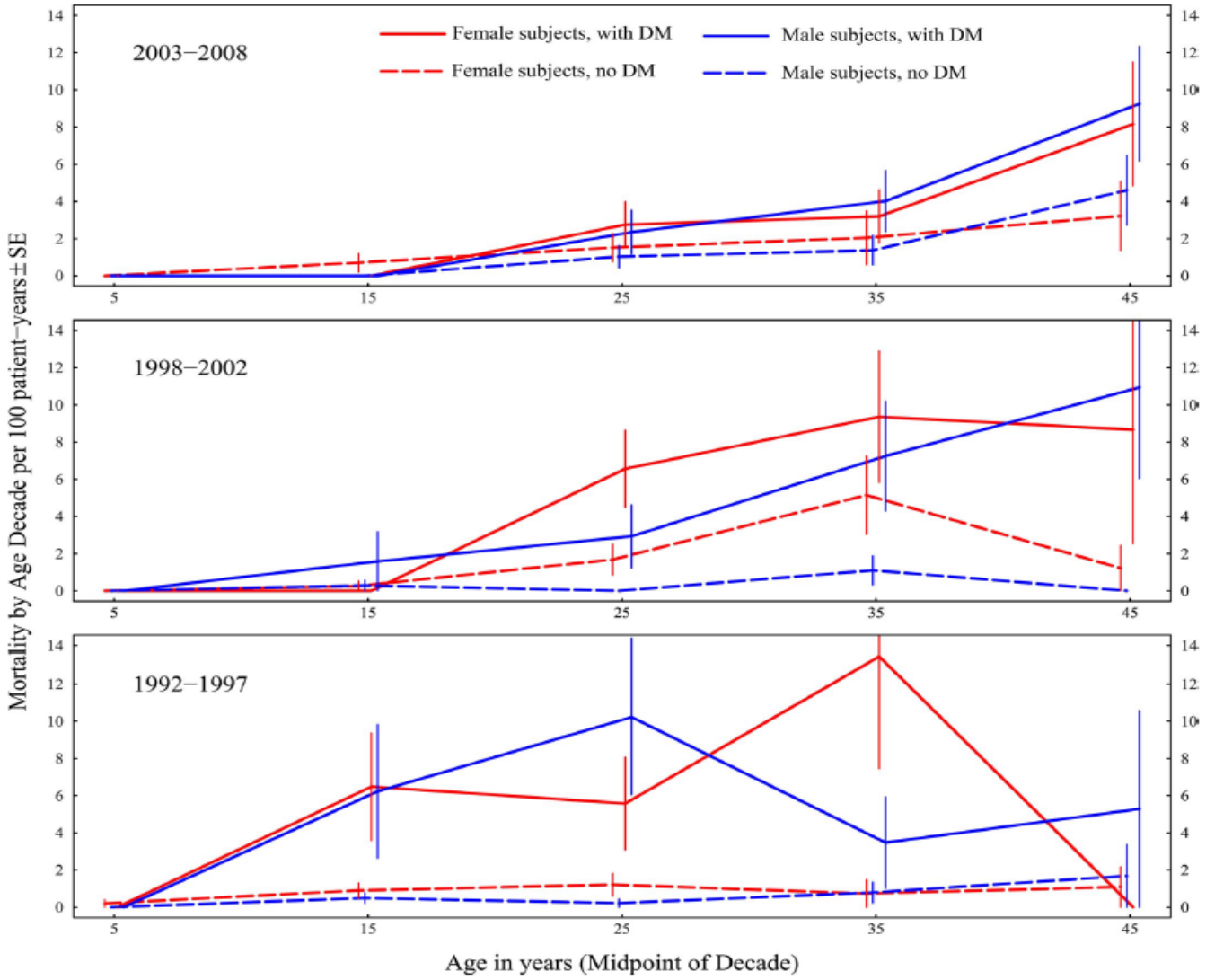
CFRD outcome - Screening



Rates of OGTT screening for CFRD have increased since the publication of the CF Foundation CFRD guidelines[40] in 2010.

CF Foundation, 2015

Moran et al, 2009



Diabetes Screening ? Improved disparities in mortality

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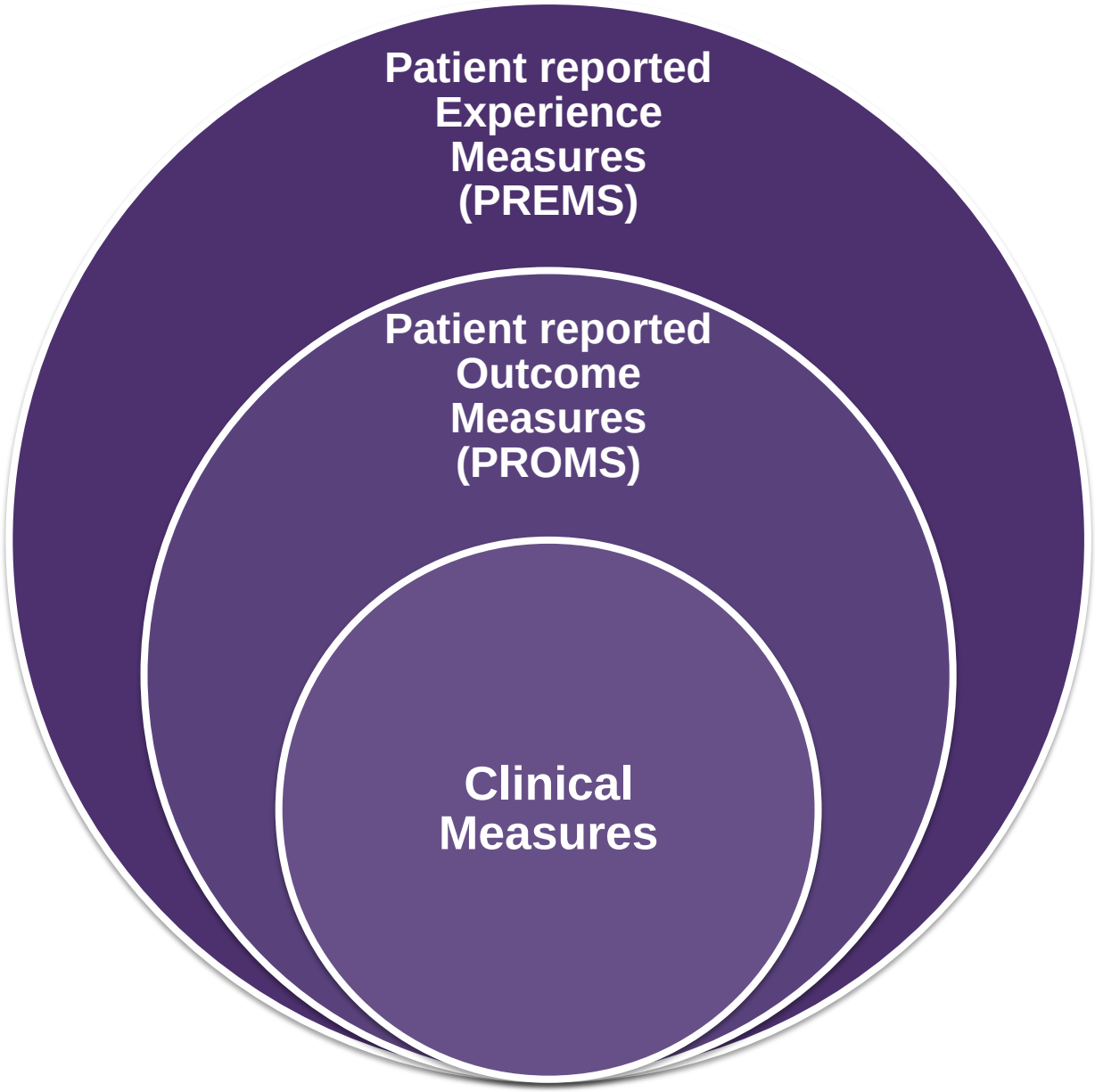
Cystic fibrosis related diabetes outcomes

- Evidence for target HbA_{1C}?

Evidence	Nutritional outcome	Impact
Clinical Care Guidelines for Cystic fibrosis - related diabetes (Moran et al, 2010)	HbA1C <7.0%	to reduce microvascular complications
Sputum glucose and glycaemic control in CFRD: a cross-sectional study (Sambeek et al, 2015)	HbA1C<6.5%	to reduce pulmonary exacerbations


Patient reported Experience Measures (PREMS)
Patient reported Outcome Measures (PROMS)
Clinical Measures

Nutrition and patient reported outcomes

Study	Nutritional indicator	Impact
Abbott et al, 2007	Enteral tube feeding	Poorer body image
Rufenacht et al, 2010	Nutrition Counselling	Improved nutrient intake and QoL
Schoff et al, 2013	Height and BMI	Positively associated with eating disturbance , physical function and body image
Abbott et al., 2015	Decreased BMI	Results in reduced body image HRQoL

Validated measures CFQ-R (Age specific) and CFQoL

FDA (USA) and NICE (UK) advocate use in evaluating new treatments

Practical and easy measures

- Digital era
- Exploration, validation & capture of simple, global, patient reported outcome measures
- European Medicines Agency (2012)

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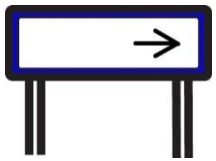
Patient experience measures (NICE 2012)



- Communication skills
- Patient awareness of roles & responsibilities
- Given opportunities to air concerns & queries
- Understanding of treatment options
- Shared decision making
- Supporting patient choice
- Able to ask for a second opinion
- Healthcare services tailored to the individuaal
- Physical & Psychological needs
- Continuity of care
- Coordinated care through information exchange
- Sharing information with partners and carers
- Contact numbers and follow-up

Article

chronic illness

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Patient experience in cystic fibrosis care: Development of a disease-specific questionnaire

Katja Stahl,[1,2] Gratiana Steinkamp,[2,3] Gerald Ullrich,[2,4] Wolfgang Schulz,[2,5] Silke van Koningsbruggen-Rietschel,[6] Hans-Eberhard Heuer,[7] Helmut Ellemunter[8] and Carsten Schwarz[9]

Gastrointestinal outcomes

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Journal of Cystic Fibrosis 14 (2015) 169–177

Journal of Cystic Fibrosis

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Review

Cystic fibrosis and the role of gastrointestinal outcome measures in the new era of therapeutic CFTR modulation ☆

Frank A.J.A. Bodewes [a,*], Henkjan J. Verkade [a], Jan A.J.M. Taminiau [b], Drucy Borowitz [c], Michael Wilschanski [d] Working group Cystic Fibrosis and Pancreatic Disease of the European Society for Paediatric Gastroenterology Hepatology and Nutrition (ESPGHAN)

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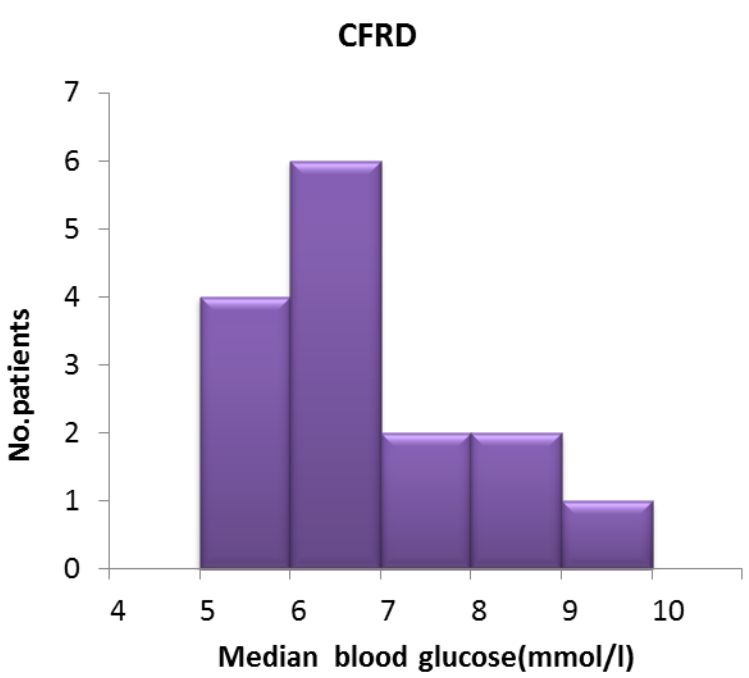
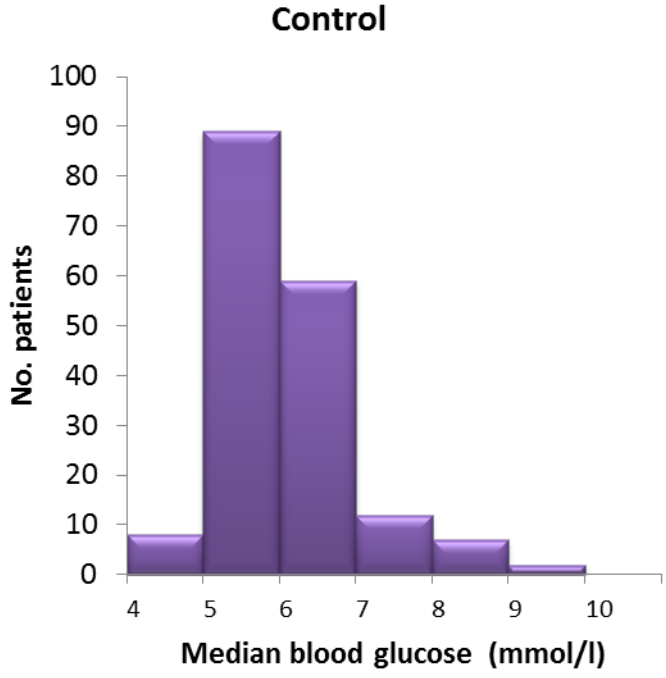
Outcome measure	Applicable to	Validated
BMI 50th percentile	All children	Yes
Weight percentile	All children	Yes
Height percentile		Yes
BMI 22	Women	Yes
BMI 23	Men	Yes
Lean tissue mass	All	Yes (DEXA)
Minimum 110% EAR Calcium intake (target levels)	All All children and adults	Less robust
Vitamin A ,D, E,K level of 30ng/ml	Children and adults with pancreatic insufficiency	Less robust
Diabetes	HbA1C <7.0%)	Early stage
PRO (M) measures	All	Yes
PRE (M) measures	All	Early stage

Use of 'real time' data in outcome measurement

Distribution of the median Random capillary blood glucose levels over a 3 year period prior to diagnosis of CFRD





White et al, (2015) Variation in random capillary blood glucose and HbA1c as predictors of Cystic Fibrosis Related Diabetes (CFRD)

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The best nutritional outcome measures

- Practical, simple, validated
- Agreed reporting of measures
- Explain all aspects of nutritional delivery and outcome
  - Class mutation
  - Comorbidity
  - Socioeconomic status
  - Improvement from baseline

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Thank you

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