Erasmus MC
University Medical Center Rotterdam

Comparing effectiveness and costs of stuttering treatment for pre-schoolers: RESTART trial

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RESTART:

Rotterdam **E**valuation study of **S**tuttering
Treatment in children
A **R**andomised **T**rial

RESTART Study Project Team

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Outline of the presentation

- Rationale for & design of the RESTART study
- Results clinical outcome and cost-effectiveness
- Conclusions

- Time for questions and discussion

Rationale for the RESTART study

- Demands and Capacities Model based treatment (Riley & Riley, 1979; Gregory & Hill, 1980; Starkweather et al, 1990; Conture, 1982) has been the standard in the Netherlands since eighties previous century
- Encouraging publications on the Lidcombe Program (e.g. Onslow et al., 1990; Lincoln & Onslow, 1997) led to introduction of LP in the Netherlands in 2000
- The Lidcombe Program offers the best evidence for an effective intervention for preschool children who stutter- this applies for children under six years of age (Nye et al, 2012).

Main conclusions Nye et al, JSLHR 2013

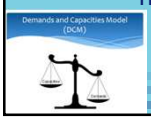
- In the **limited data** available, at this time, the Lidcombe Program offers the best evidence for an effective intervention for preschool children who stutter.
- This is not to say that other approaches may not be effective, but the **available data** only allow us to conclude that there is insufficient information employing the highest research standards in the discipline
- The findings for those studies comparing two treatment groups suggest that, while the intervention may result in a positive effect, the result may be no greater for one type of intervention than for the other

In addition

- no independent replications
- no (prospective) long-term follow-up results
- no cost-effectiveness data comparing the LP to other treatments

DCM based treatment

- Approach will address the child and the environment
- Hypotheses about changes in environmental and child factors – decreasing demands and improving capacities - are put forward
- Aim: restore the favorable balance between Demands and Capacities, resulting in normally fluent speech



Lidcombe Program

- Child directed approach
- Based on behavioral principles
- Fluency is targeted by using operant conditioning (using praise and mild corrections- in a ratio of at least 5 : 1), going from structured to unstructured situations
- Aim: fluent speech

Aim of the RESTART study

To compare the effectiveness and cost-effectiveness of the Lidcombe Program with DCM based treatment

The ideal study design

Three treatment arms: (1) LP (2) DCM (3) Placebo or no treatment

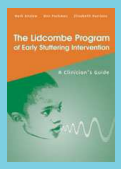
However:

- Effectiveness of LP after 9 months has been shown: **unethical** to delay treatment for a longer period;
- Chance for full recovery diminishes after 15 months since onset;
- Spontaneous recovery takes **many** years;
- Placebo is not realistic in stuttering therapy;
- Relevance of study for decision makers in the Netherlands: compare a new treatment (LP) to a current standard (DCM).

Design of the RESTART study

- Randomised comparative trial, with cost-effectiveness analysis
- Two treatments:

  - Lidcombe Program (LP)
  Onslow, Packman & Harisson (2003)



  - RESTART Demands and Capacities Model based treatment
  http://www.nedverstottertherapie.nl/pdf/RESTART-DCM.Method.incl.bijlagen04-12-11.pdf

- Power calculation based on 15% difference in recovery: n=196

In- and exclusion criteria

+ age 3.0-6.3 years
+ stuttering severity rating of at least 2 ('mild') on 8-point scale (Yairi & Ambrose, 2005) by parent and clinician
+ stuttering frequency at least **3%** (syllables)
+ stuttering for at least **6** months

- diagnosis of an emotional, behavioral, learning or neurological disorder
- a lack of proficiency in Dutch for children or parents

Randomisation

- gender (male, female)
- family history of persistency and recovery
- severity of stuttering (SSI- mild, moderate, severe)
- time since onset (6-12 months, 12-18 months, 19+ months)
- prior treatment for stuttering
- SLT

Outcome measures

Primary outcome
percentage of recovered children at 18 M

Secondary outcomes

- frequency of stuttering (%SS)
- Severity rating by parent
- Health related Quality of Life (EQ-VAS)
- Child Behavior Checklist
- KiddyCAT
- Severity rating by SLT
- Severity rating by child

Moments of measurement

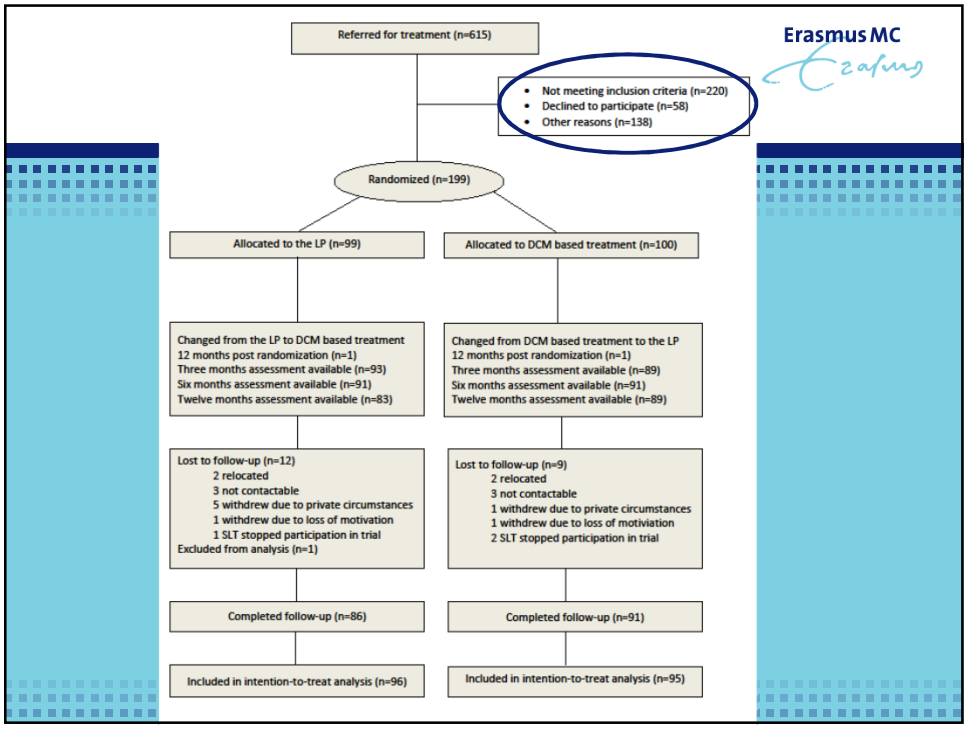
Lidcombe Programme

Baseline 3 mnd 6 mnd 9 mnd 12 mnd **18 mnd**

RESTART-DCM based treatment

How to define if a child stopped stuttering?

- Defined as less than 1.5% SS (cf Clark et al. 2013)
- Based on three recordings of 10-15 minutes in a period of two weeks:
  * child speaking to a parent at home
  * child speaking to a non-family member at home
  * child speaking to a non-family member away from home
  (Ingham 1998; Jones, et al. 2005; Sawyer and Yairi 2006)
- Blind evaluation of speech samples (2709!)


Erasmus MC
Referred for treatment (n=615)
Not meeting inclusion criteria (n=220)
Declined to participate (n=58)
Other reasons (n=138)
Randomized (n=199)
Allocated to the LP (n=99)
Allocated to DCM based treatment (n=100)
Changed from the LP to DCM based treatment
12 months post randomization (n=1)
Three months assessment available (n=93)
Six months assessment available (n=91)
Twelve months assessment available (n=83)
Changed from DCM based treatment to the LP
12 months post randomization (n=1)
Three months assessment available (n=89)
Six months assessment available (n=91)
Twelve months assessment available (n=89)
Lost to follow-up (n=12)
2 relocated
3 not contactable
5 withdrew due to private circumstances
1 withdrew due to loss of motivation
1 SLT stopped participation in trial
Excluded from analysis (n=1)
Lost to follow-up (n=9)
2 relocated
3 not contactable
1 withdrew due to private circumstances
1 withdrew due to loss of motiviation
2 SLT stopped participation in trial
Completed follow-up (n=86)
Completed follow-up (n=91)
Included in intention-to-treat analysis (n=96)
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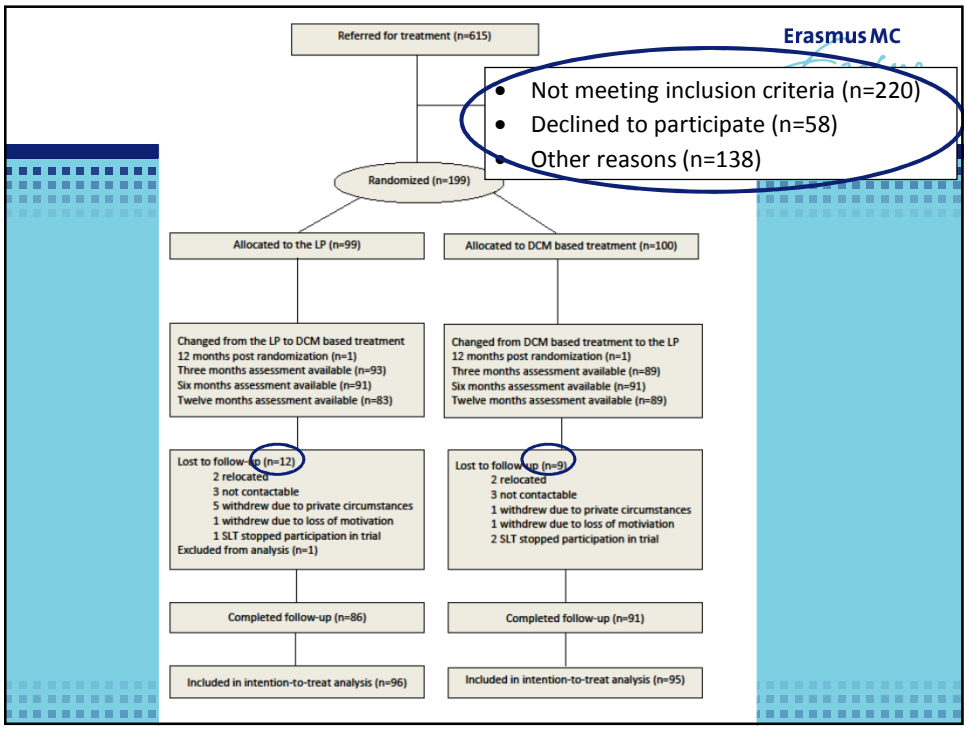
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Results after 18 months: % recovered children

Therapy	% recovered
Lidcombe Program	76.5%
RESTART DCM	71.4%

Not significant

Therapy * severity
Therapy * TSO
Therapy * age

Not significant

Results after 18 months: % recovery for severity groups

	DCM	DCM	DCM	LP	LP	LP
	SSI-mild	SSI-moderate	SSI-severe	SSI-mild	SSI-moderate	SSI-severe
Recovery (%)	72.4	69.6	75.0	89.7	74.4	58.8

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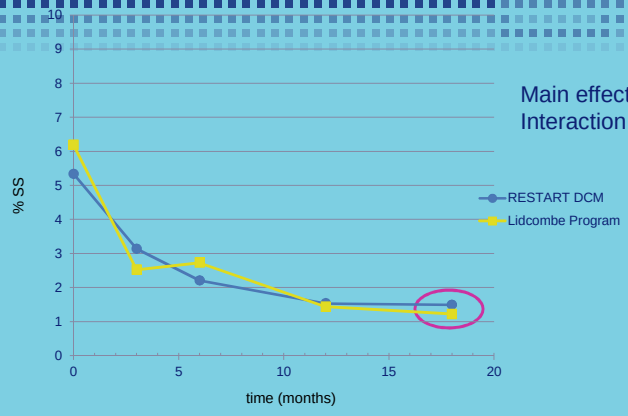
Not significant

% Syllables Stuttered

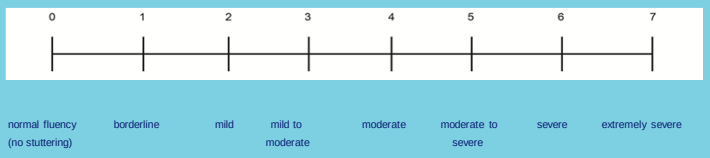


Main effect time (*p*=.002)
Interaction time*therapy (*p*=.008)

% Syllables Stuttered at 18 months

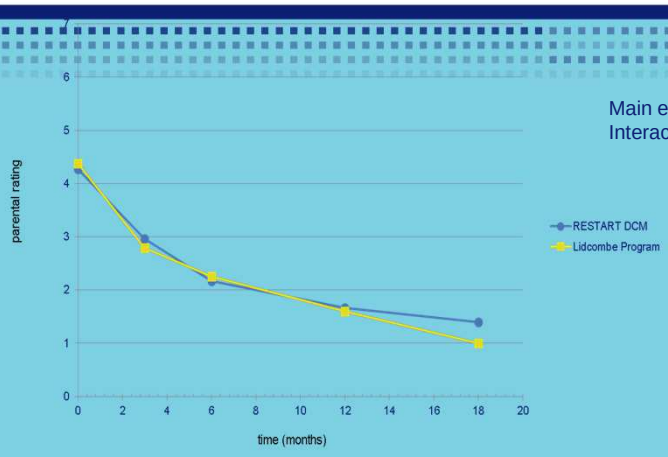
	Baseline (n=197)	18 months (n=176)
Lidcombe Program	6.2 (4.4)	1.2 (2.1)
RESTART DCM	5.3 (4.3)	1.5 (2.1)

Parental rating of stuttering severity



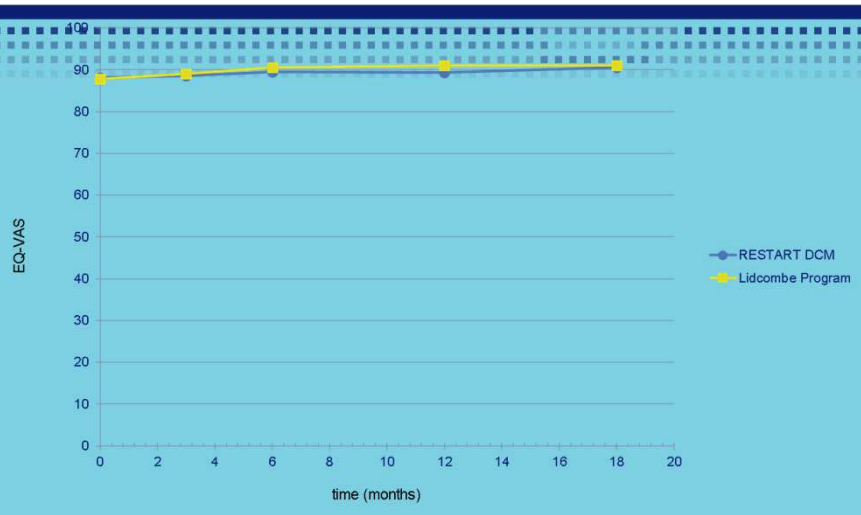
Yairi & Ambrose (2005)

Parental rating of stuttering severity



Main effect time (p <.001)
Interaction time*therapy (p <.001)

EQ VAS



Child Behaviour Check List (CBCL)

- Emotional and behavioral problems (Achenbach, 2000)

	CBCL Internal		CBCL External		CBCL Total problems	
	baseline	18 months	baseline	18 months	baseline	18 months
Lidcombe Program	10.4 (7.9)	5.5 (5.2)	13.6 (7.4)	7.1 (5.8)	36.2 (20.6)	21.8 (15.4)
RESTART DCM	7.4 (5.9)	4.2 (4.5)	11.2 (7.6)	6.2 (5.7)	27.9 (17.6)	18.6 (13.8)

KiddyCAT

- Speech attitude of the child (Vanryckeghem, 2005)

	KiddyCAT	
	Baseline (n=182)	18 months (n=116)
Lidcombe Program	3.6 (2.5)	1.2 (1.5)
RESTART DCM	3.9 (2.9)	2.0 (2.1)

CAT – D data 18 months post onset treatment by treatment (Communications ATtitudes – Dutch)

		Score (mean, SD)
DCM	CAT (n=21)	9.1 (6.6)
Lidcombe	CAT (n=10)	9.9 (9.1)

Severity rating child at 18 months

- Rating by the child on a 4-point scale:

  1= I do not stutter anymore

	Severity rating child
Lidcombe Program (n=81)	1.4 (0.5)
RESTART DCM (n=87)	1.4 (0.5)

Severity rating Speech Language Therapist at 18 months

- Rating by the SLT on 8-point scale (Yairi, 2005):

  0= normally fluent

  1= borderline

  2= mild stuttering

	Severity rating SLT
Lidcombe Program (n=82)	1.1 (1.4)
RESTART DCM (n=86)	1.4 (1.4)

Summary clinical results

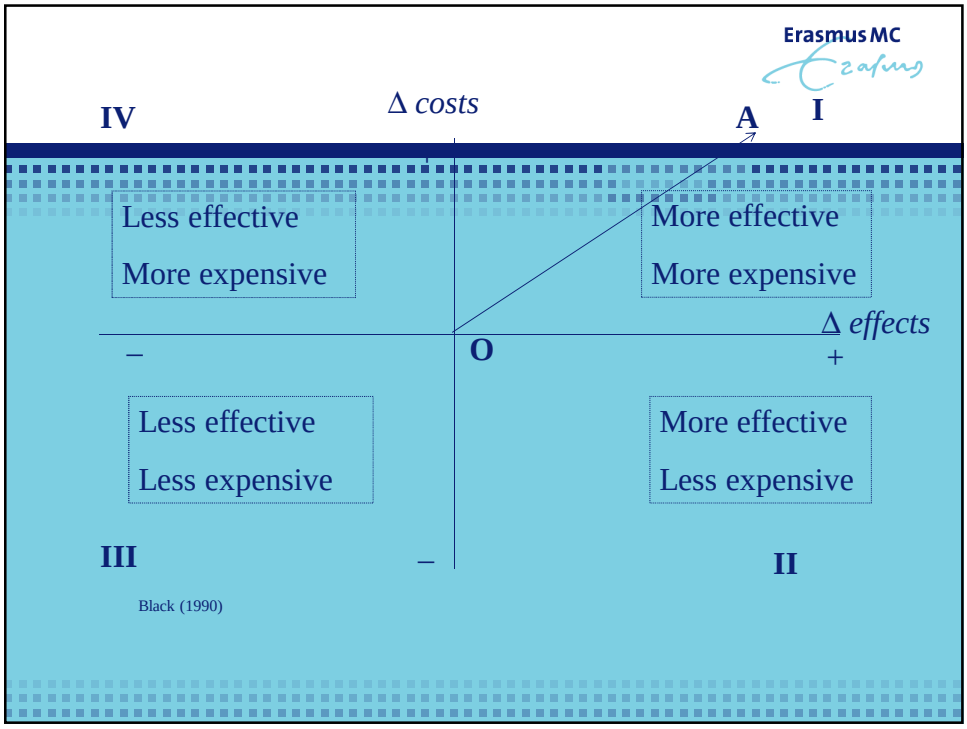
- No significant difference in % recovery and %SS after 18 months;
- However, significant *lower* %SS for children in the LP after three months;
- No significant difference on other outcome measures;
- Slightly better scores for LP on most outcome measures, but none of them being significant.

Cost effectiveness

- Maximizing health outcomes per Euro spend
  "Value for money"
- Cost effectiveness ratio = $\frac{\text{costs LP} - \text{costs DCM}}{\text{effects LP} - \text{effects DCM}}$

* Extra costs per recovered child

* Extra costs per QALY *(quality adjusted life year)*
-> HUI3 (children aged 5-6)
-> EQ-VAS (all children)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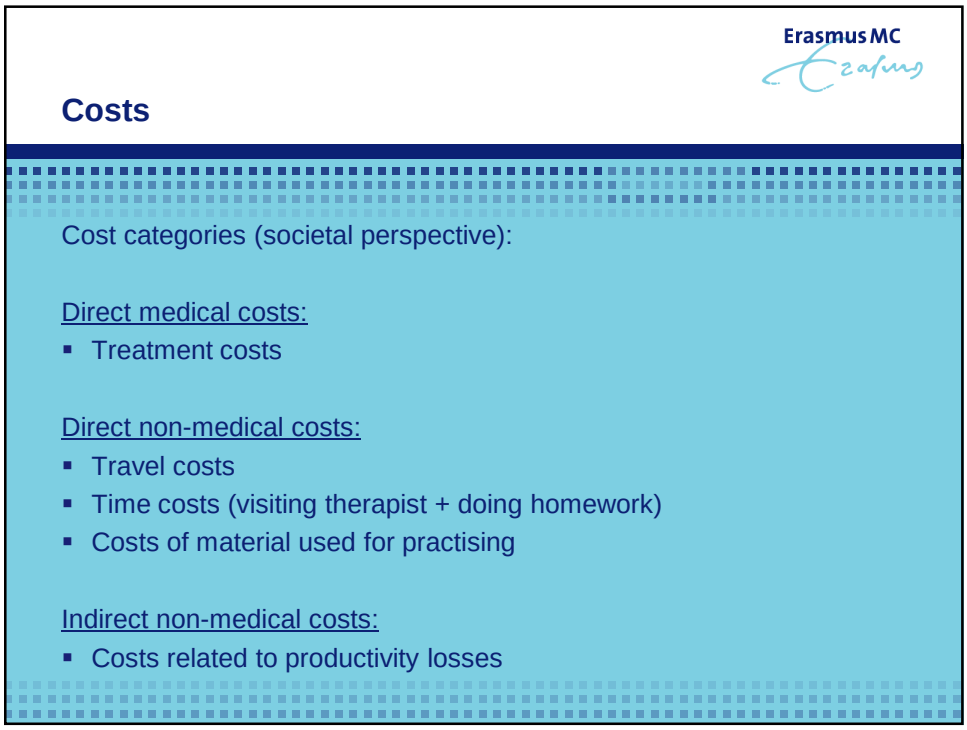
IV
Δ costs
A
I
Less effective
More expensive
More effective
More expensive
Δ effects
–
O
+
Less effective
Less expensive
More effective
Less expensive
III
–
II
Black (1990)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Costs
Cost categories (societal perspective):
Direct medical costs:
Treatment costs
Direct non-medical costs:
Travel costs
Time costs (visiting therapist + doing homework)
Costs of material used for practising
Indirect non-medical costs:
Costs related to productivity losses

Costs

Direct medical costs:

- Treatment costs: DCM € 1287; LP € 1422

Direct non-medical costs:

- Travel costs
- Time costs (visiting therapist + doing homework)
- Costs of material used for practising

Indirect non-medical costs:

- Costs related to productivity losses

Total costs: DCM: € 3032 ; LP: € 3199

Treatment time

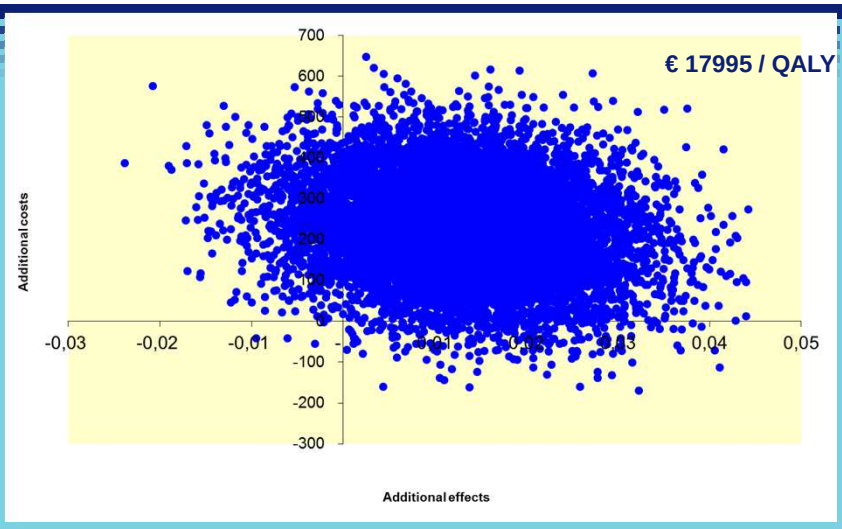
Number of treatment sessions RESTART DCM: 19.5 (SD 10.3)

Number of treatment hours: 18

Number of treatment sessions LP: 22.2 (SD 11.2)

Number of treatment hours: 20

Cost-effectiveness plane (HUI3)



Conclusions for Speech Language Therapists

- No evidence for the LP being more effective than RESTART DCM 18 months post start treatment
- Results on CBCL, KiddyCAT and Bristol questionnaire at 18 months show that neither therapies do negatively affect the child's behaviour or attitude

Conclusions for Children & Parents

- At 18 months post start treatment no significant difference in outcomes
- At 3 months post start treatment better outcomes for LP – might play a role in motivation of parents
- Preference of parent and/or SLT could be leading in therapy choice

Conclusion for Policy makers

- Results do not indicate a clear preference with regard to reimbursement

Main conclusions Nye et al, JSLHR 2013

- In the **limited data** available,(…)
- This is not to say that other approaches may not be effective, but the **available data (…)**
- The findings for those studies comparing two treatment groups suggest that, while the intervention may result in a positive effect,

the result may be no greater for one type of intervention than for the other

TRAINING RESTART DCM

WORKSHOP *DEMANDS & CAPACITIES MODEL* (RESTART)
FOR TREATMENT OF PRE-SCHOOL CHILDREN WHO STUTTER - 3 DAYS

July 14-15-16, 2016
Rotterdam, Erasmus MC
The Netherlands

Marie-Christine Franken PhD & Ellen Laroes

Contact restartdcm@erasmusmc.nl

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Thank you for your attention!