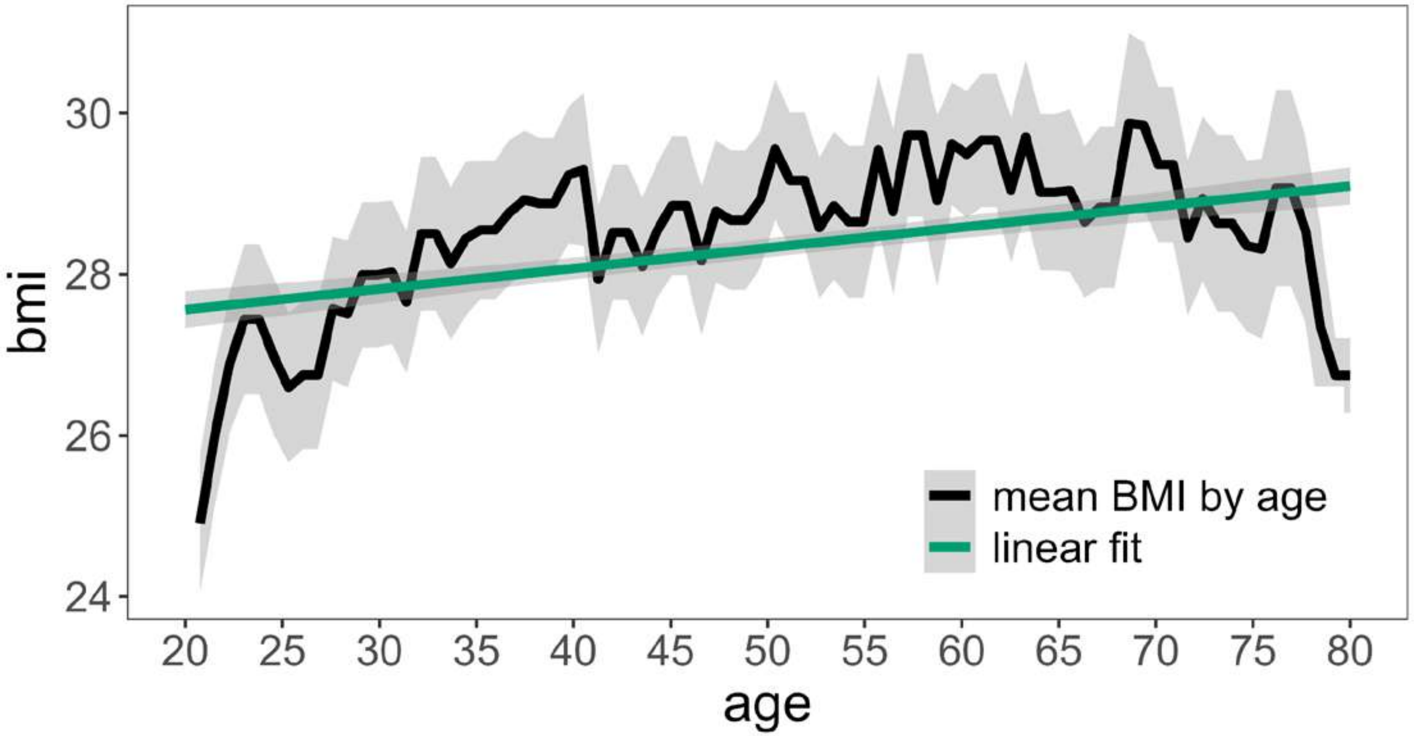
A categorization and comparison of performance measures for estimated non-linear associations with an outcome

Theresa Ullmann, Georg Heinze, Michal Abrahamowicz, Aris Perperoglou, Willi Sauerbrei, Matthias Schmid, Daniela Dunkler, for TG2 of the STRATOS initative

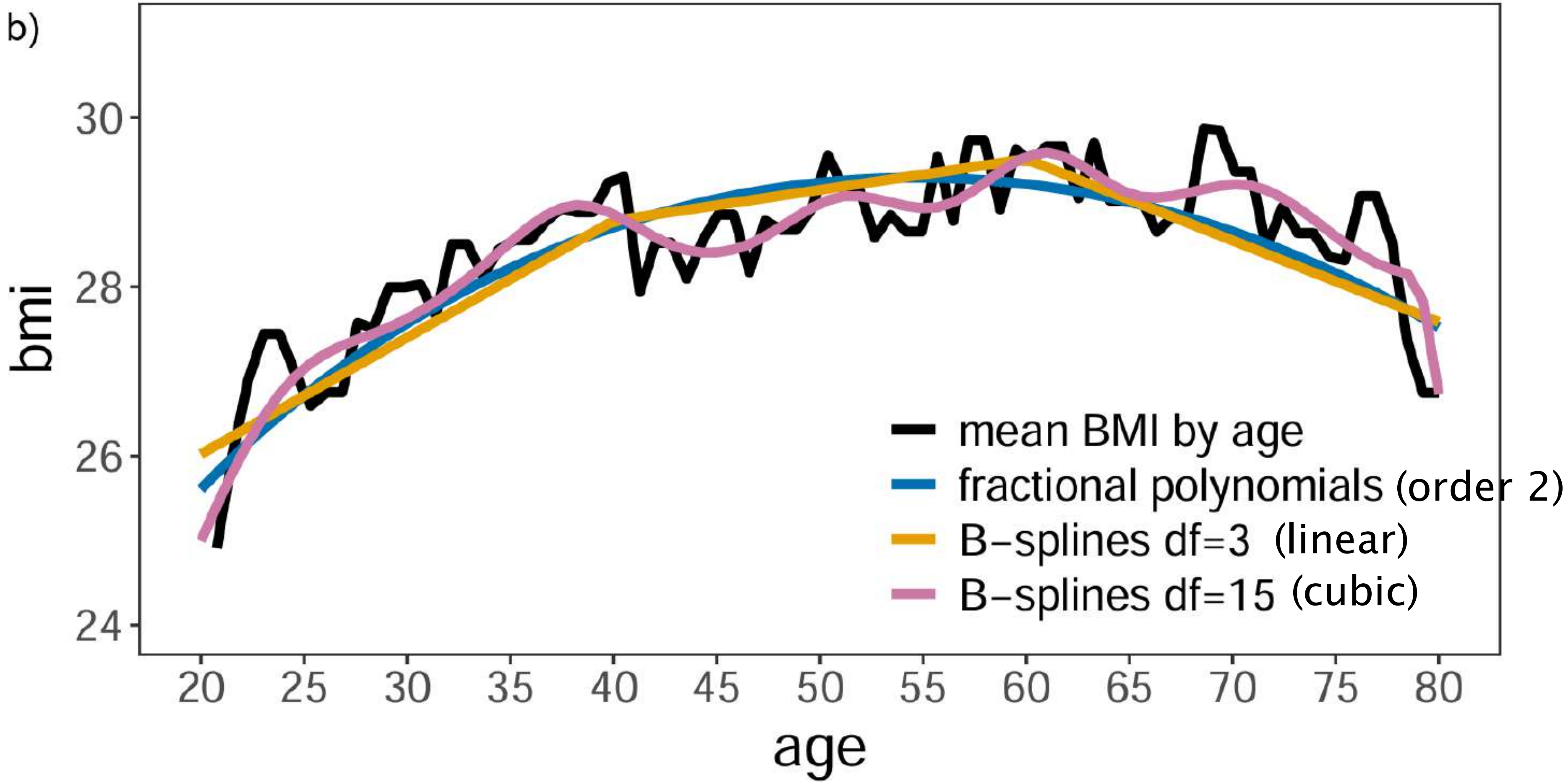
Presenter: Georg Heinze
Institute of Clinical Biometrics, Center for Medical Data Science, Medical University of Vienna

Background & motivation

- Consider the association of BMI with age (NHANES)
- How to separate systematic from unsystematic variation?
- Linear model probably a poor smoother

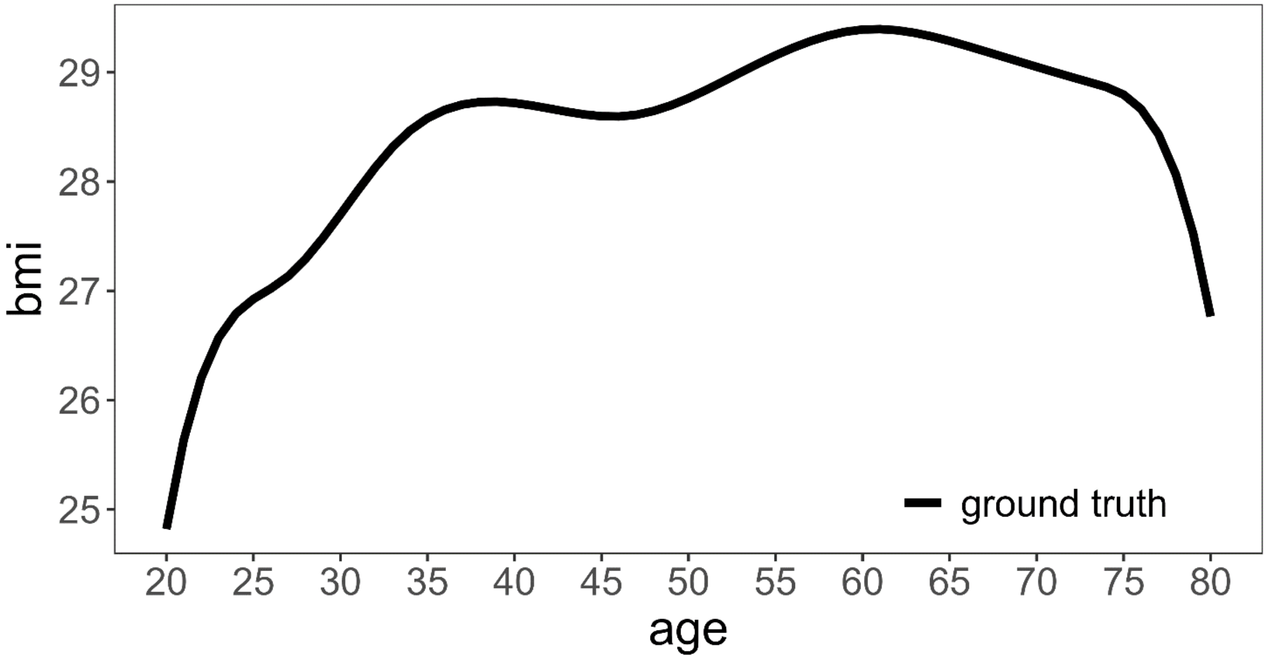


More smoothers...



A simulation study to compare methods?

- Aim: to compare the performance of different methods of nonlinear modeling
- Data generation mechanism:

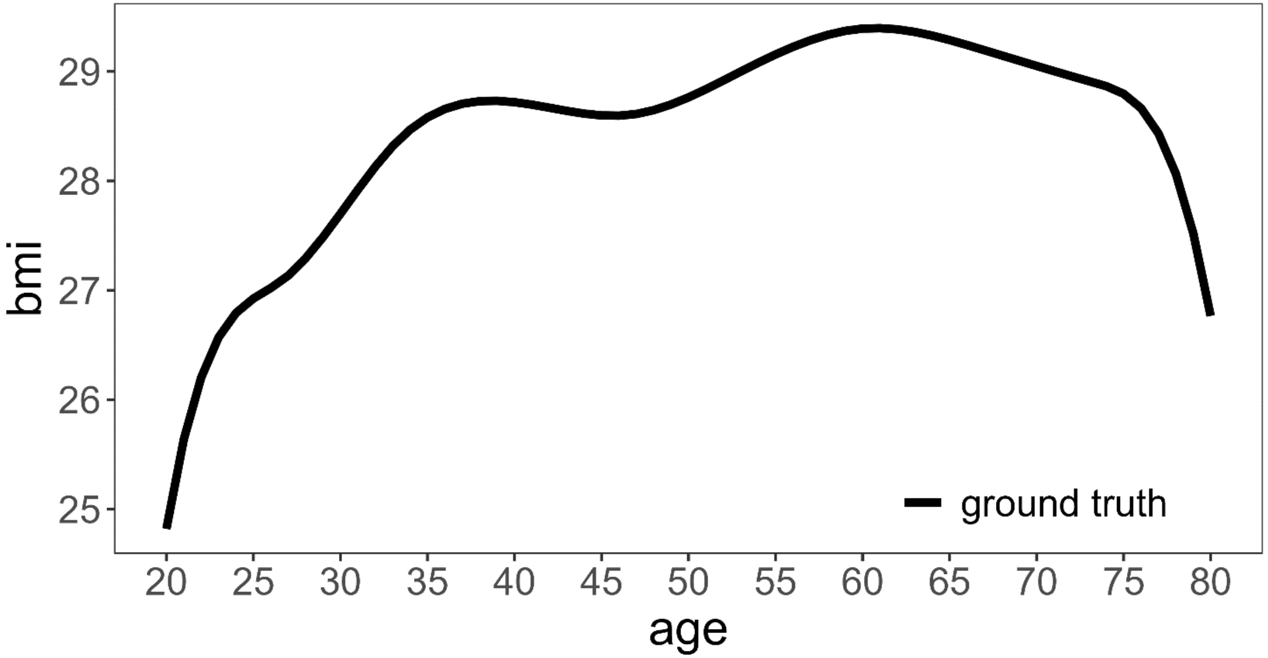


- Estimand: predicted BMI

ADEMP: Morris et al, StatMed 2019

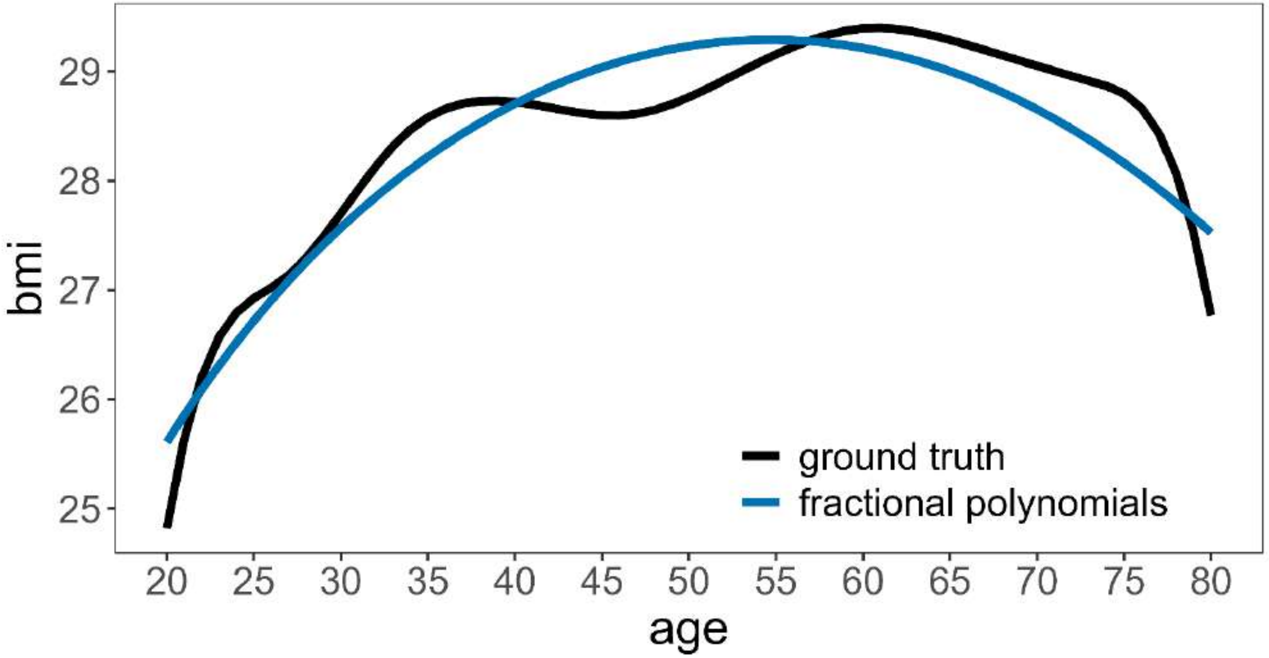
A simulation study to compare methods?

- Methods:



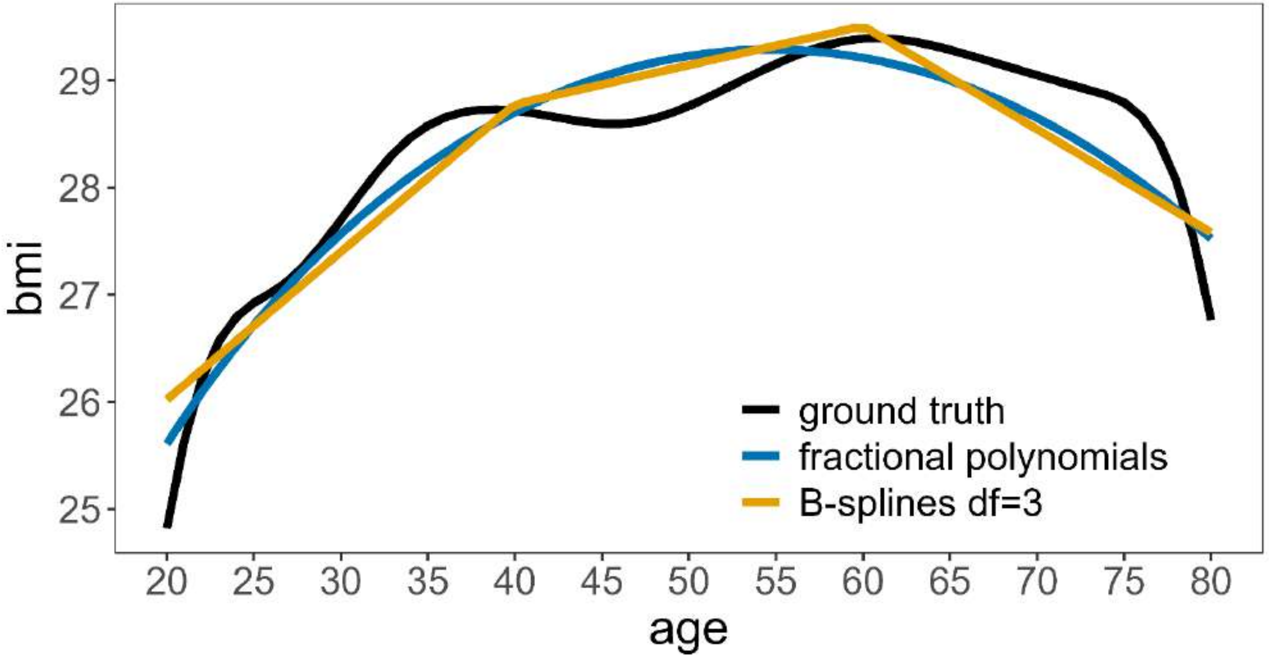
A simulation study to compare methods?

- Methods:



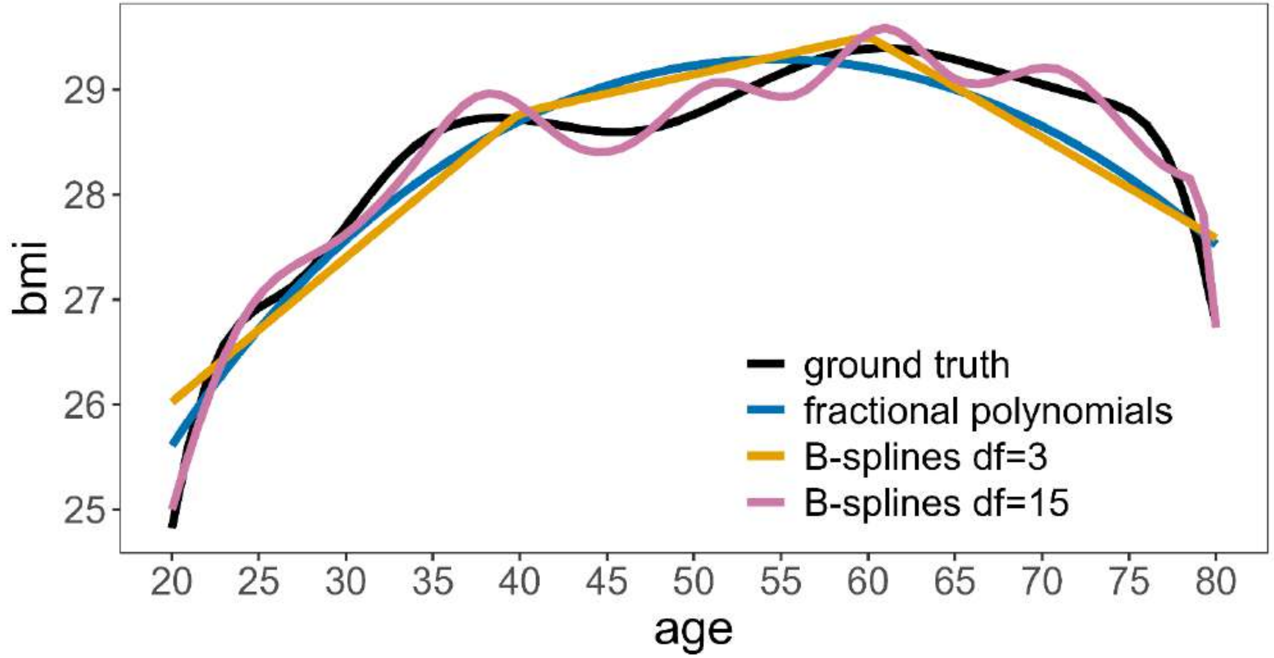
A simulation study to compare methods?

- Methods:



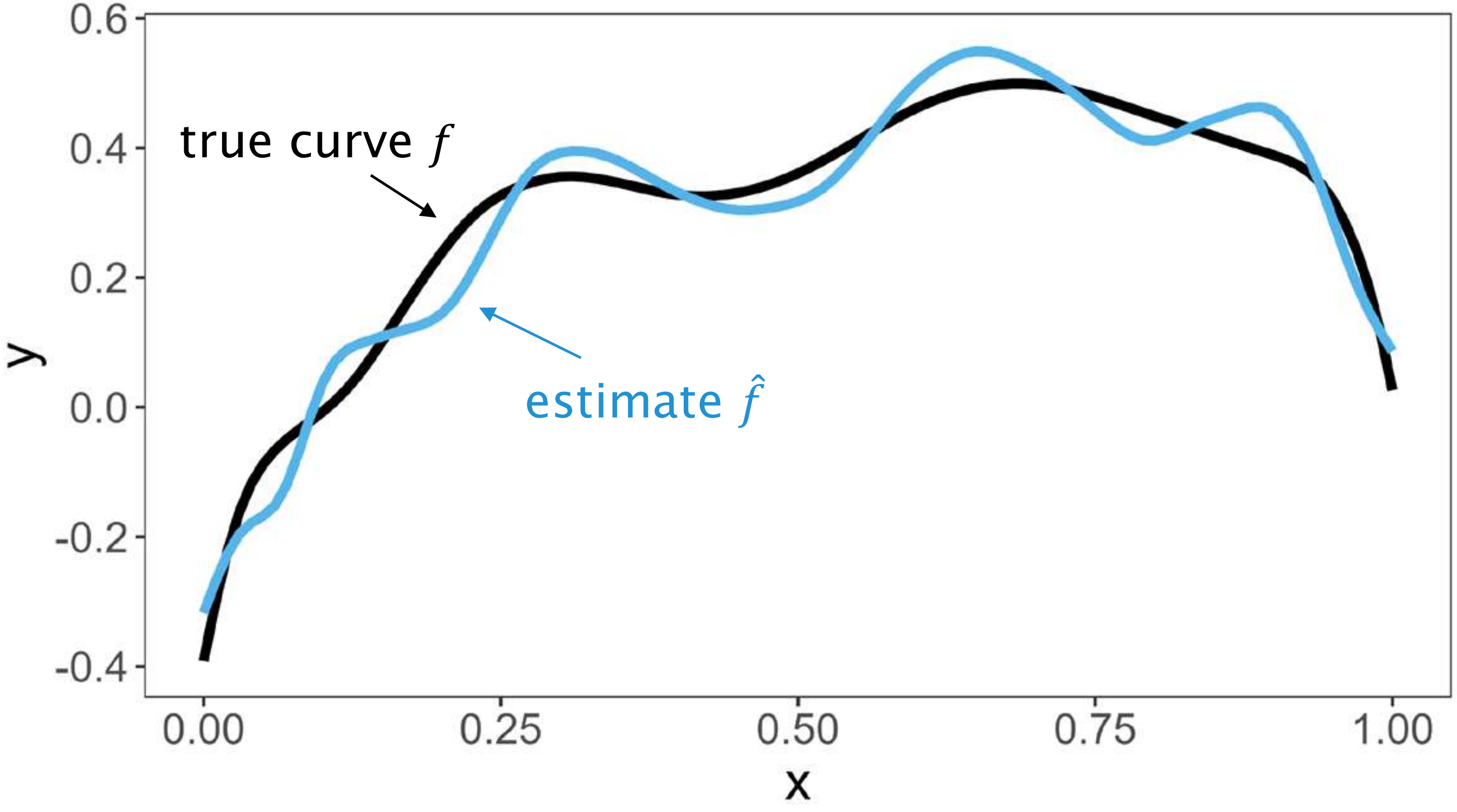
A simulation study to compare methods?

- Methods:



A simulation study to compare methods?

- Performance measures: compare estimated with true curve



A simulation study to compare methods?

- Performance measures:

$$\int_{F_X^{-1}(0.01)}^{F_X^{-1}(0.99)} |\hat{f}(x) - f(x)|\hat{p}(x)\mathrm{d}x$$

Buchholz et al. (2014) (see also Govindarajulu et al., 2007)

A simulation study to compare methods?

- Performance measures:

$$\int_{F_X^{-1}(0.01)}^{F_X^{-1}(0.99)} |\hat{f}(x) - f(x)|\hat{p}(x)\mathrm{d}x$$

Buchholz et al. (2014) (see also Govindarajulu et al., 2007)

$$\int_{F_X^{-1}(0.05)}^{F_X^{-1}(0.95)} \left(\hat{f}'(x) - f'(x)\right)^2 \mathrm{d}F_X(x)$$

Binder et al. (2011)

A simulation study to compare methods?

- Performance measures:

Region of interest: 1st to 99th percentile of F_X

$$\int_{F_X^{-1}(0.01)}^{F_X^{-1}(0.99)} |\hat{f}(x) - f(x)|\hat{p}(x)\mathrm{d}x$$

Buchholz et al. (2014) (see also Govindarajulu et al., 2007)

Region of interest: 5th to 95th percentile of F_X

$$\int_{F_X^{-1}(0.05)}^{F_X^{-1}(0.95)} \left(\hat{f}'(x) - f'(x)\right)^2 \mathrm{d}F_X(x)$$

Binder et al. (2011)

A simulation study to compare methods?

- Performance measures:

Absolute loss

$$\int_{F_X^{-1}(0.01)}^{F_X^{-1}(0.99)} |\hat{f}(x) - f(x)|\hat{p}(x)\mathrm{d}x$$

Buchholz et al. (2014) (see also Govindarajulu et al., 2007)

$$\int_{F_X^{-1}(0.05)}^{F_X^{-1}(0.95)} \left(\hat{f}'(x) - f'(x)\right)^2 \mathrm{d}F_X(x)$$

Binder et al. (2011)

Quadratic loss

A simulation study to compare methods?

- Performance measures:

function

$$\int_{F_X^{-1}(0.01)}^{F_X^{-1}(0.99)} |\hat{f}(x) - f(x)|\hat{p}(x)\mathrm{d}x$$

Buchholz et al. (2014) (see also Govindarajulu et al., 2007)

$$\int_{F_X^{-1}(0.05)}^{F_X^{-1}(0.95)} \left(\hat{f}'(x) - f'(x)\right)^2 \mathrm{d}F_X(x)$$

Binder et al. (2011)

first derivative

A simulation study to compare methods?

- Performance measures:

Integral weighted with precision

$$\int_{F_X^{-1}(0.01)}^{F_X^{-1}(0.99)} |\hat{f}(x) - f(x)|\hat{p}(x)\mathrm{d}x$$

Buchholz et al. (2014) (see also Govindarajulu et al., 2007)

$$\int_{F_X^{-1}(0.05)}^{F_X^{-1}(0.95)} \left(\hat{f}'(x) - f'(x)\right)^2 \mathrm{d}F_X(x)$$

Binder et al. (2011)

Integral over distribution of X

A simulation study to compare methods?

- Performance measures:

Region of interest × Loss × Functional characteristic × Aggregation × …

A simulation study to compare methods?

- Performance measures:

?

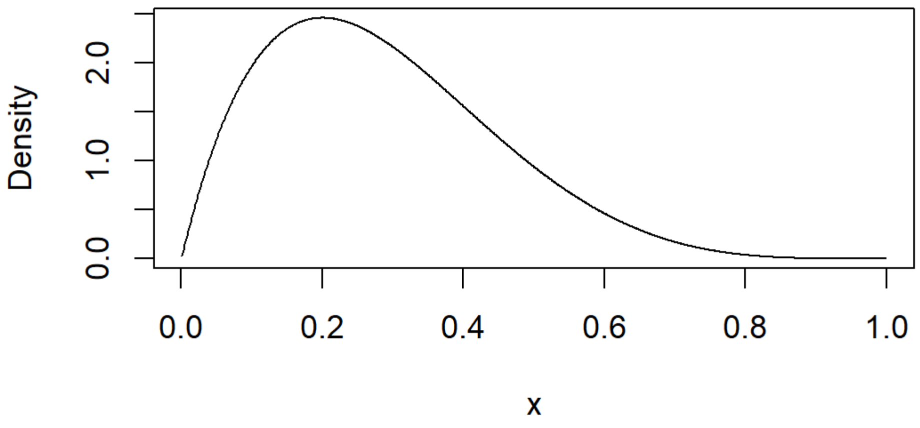
Region of interest × Loss × Function characteristic × Aggregation × …

Aims of this project

- To provide a comprehensive characterization of performance measures to be used in methods comparison studies
  - Define aspects of such measures
  - Suggest sensible combinations of choices for each of the aspects
- To demonstrate with simple illustrative examples and some hypothetical ‚methods‘
  - How the resulting performance measures behave
  - That different performance measures capture different aspects of behaviour

The aspects:

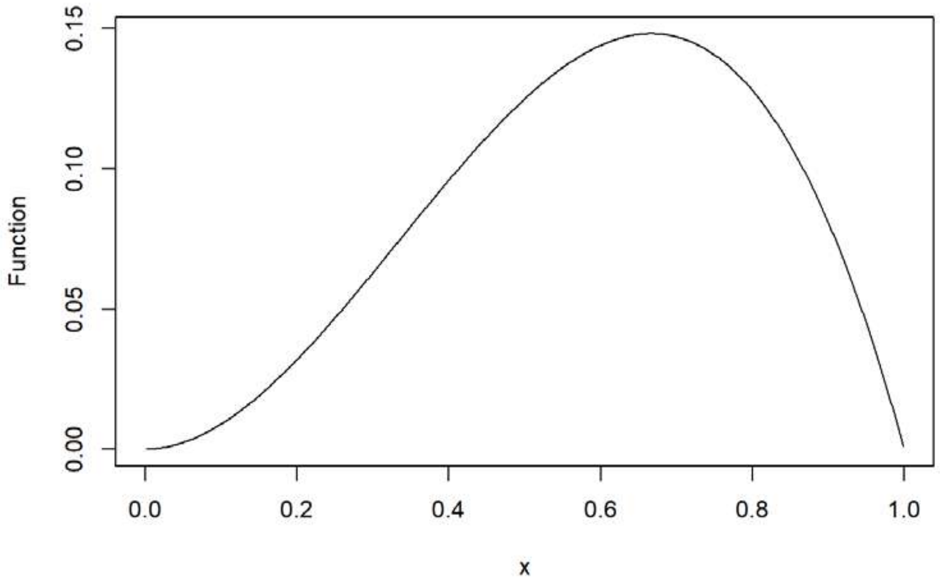
- Localization: Where are we looking at?

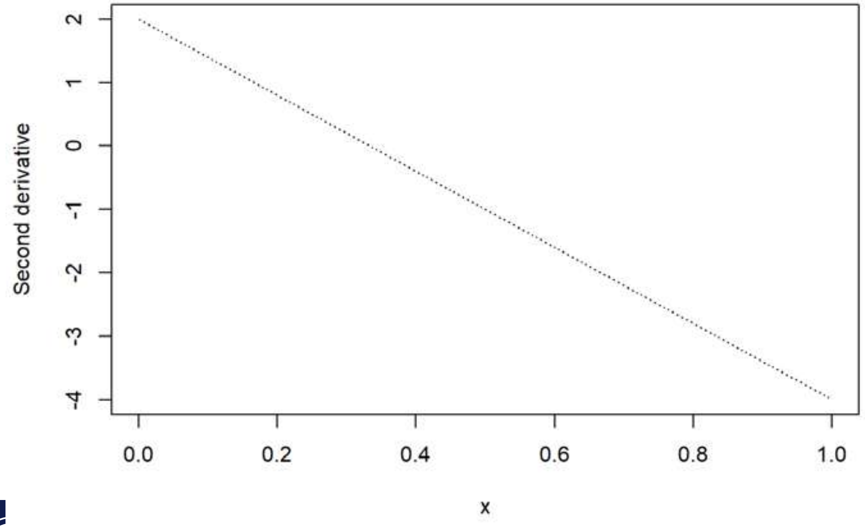


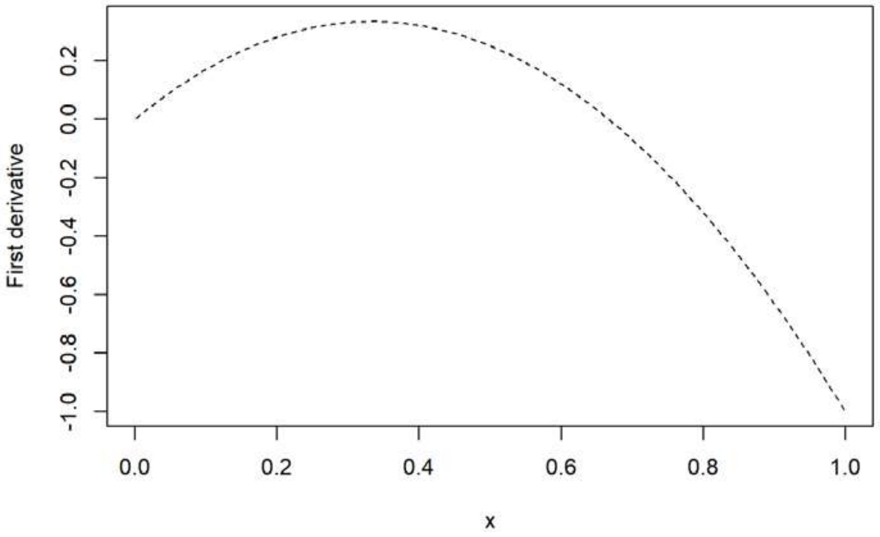
  - The full range of values (global)
  - A subrange (region)
  - A single value (point)

The aspects:

- Functional characteristic:
  - The function itself
  - First derivative
  - Second derivative







The aspects:

- Type of loss:
  - Difference: $m(x) = \hat{f}(x) - f(x)$
  - Absolute difference: $m(x) = |\hat{f}(x) - f(x)|$
  - Quadratic difference: $m(x) = \left(\hat{f}(x) - f(x)\right)^2$
  - ϵ-level accuracy: $m(x) = \mathrm{I}(\left|\hat{f}(x) - f(x)\right| \leq \epsilon)$

If we consider the range or a region:

- Axis of aggregation:
  - Y
    - Integration over dx: $\int m(x)\, dx$
    - Integration over dF(x) [=expected value): $\int m(x)\, dF(x)$
  - X
    - Location of maximum/minimum f(x) (=$argmax\left(\hat{f}(x)\right), argmin\left(\hat{f}(x)\right)$)
    - Number of roots (e.g. of $\hat{f}'(x)$)

Combining these aspects

Select the performance measure

Localization:

- [x] Range
- [ ] Point

Functional characteristic:

- [x] f(x)
- [ ] f'(x)
- [ ] f''(x)

Loss:

- [x] Difference
- [ ] Absolute
- [ ] Squared
- [ ] Epsilon-level accuracy

Axis of aggregation:

- [x] Y
- [ ] X

Type of aggregation:

- [x] Integration over dx
- [ ] Expectation over dF_X
- [ ] Quantile with respect to F_X
- [ ] Maximum
- [ ] Minimum

Scope of aggregration:

- [x] whole range $[0, 1]$
- [ ] subrange $[F_X^{-1}(0.05), F_X^{-1}(0.95)]$

$$= \int \left(\hat{f}(x) - f(x)\right) dx$$

„mean deviation“

Combining these aspects

Select the performance measure

Localization:
- (●) Range
- ( ) Point

Functional characteristic:
- (●) f(x)
- ( ) f'(x)
- ( ) f''(x)

Loss:
- ( ) Difference
- (●) Absolute
- ( ) Squared
- ( ) Epsilon-level accuracy

Axis of aggregation:
- (●) Y
- ( ) X

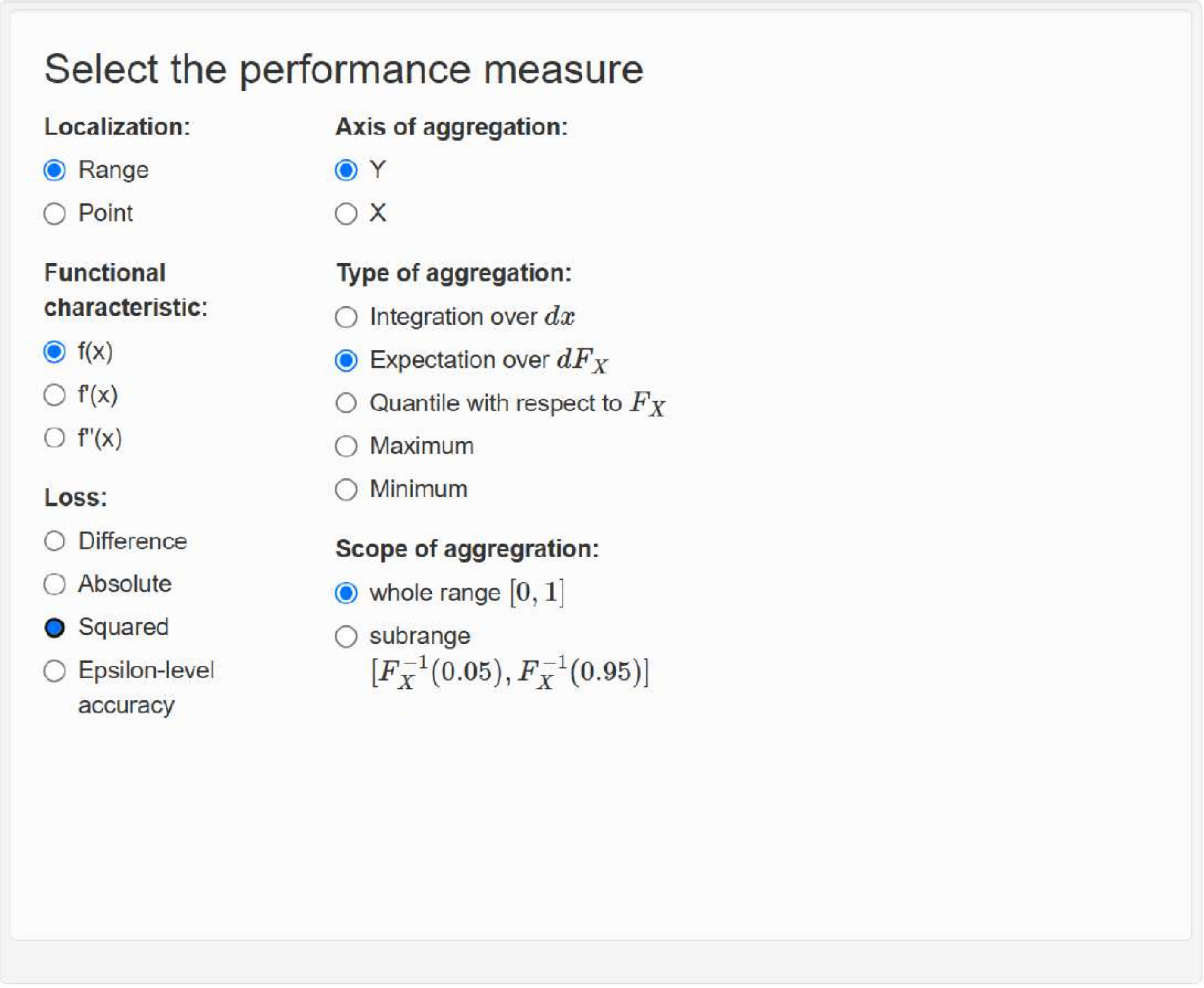
Type of aggregation:
- (●) Integration over dx
- ( ) Expectation over dF_X
- ( ) Quantile with respect to F_X
- ( ) Maximum
- ( ) Minimum

Scope of aggregration:
- (●) whole range $[0, 1]$
- ( ) subrange $[F_X^{-1}(0.05), F_X^{-1}(0.95)]$

$$=\int |\hat{f}(x) - f(x)| dx$$

„mean absolute deviation“

Combining these aspects



Select the performance measure

Localization:

- (●) Range
- ( ) Point

Functional characteristic:

- (●) f(x)
- ( ) f'(x)
- ( ) f''(x)

Loss:

- ( ) Difference
- ( ) Absolute
- (●) Squared
- ( ) Epsilon-level accuracy

Axis of aggregation:

- (●) Y
- ( ) X

Type of aggregation:

- ( ) Integration over dx
- (●) Expectation over dF_X
- ( ) Quantile with respect to F_X
- ( ) Maximum
- ( ) Minimum

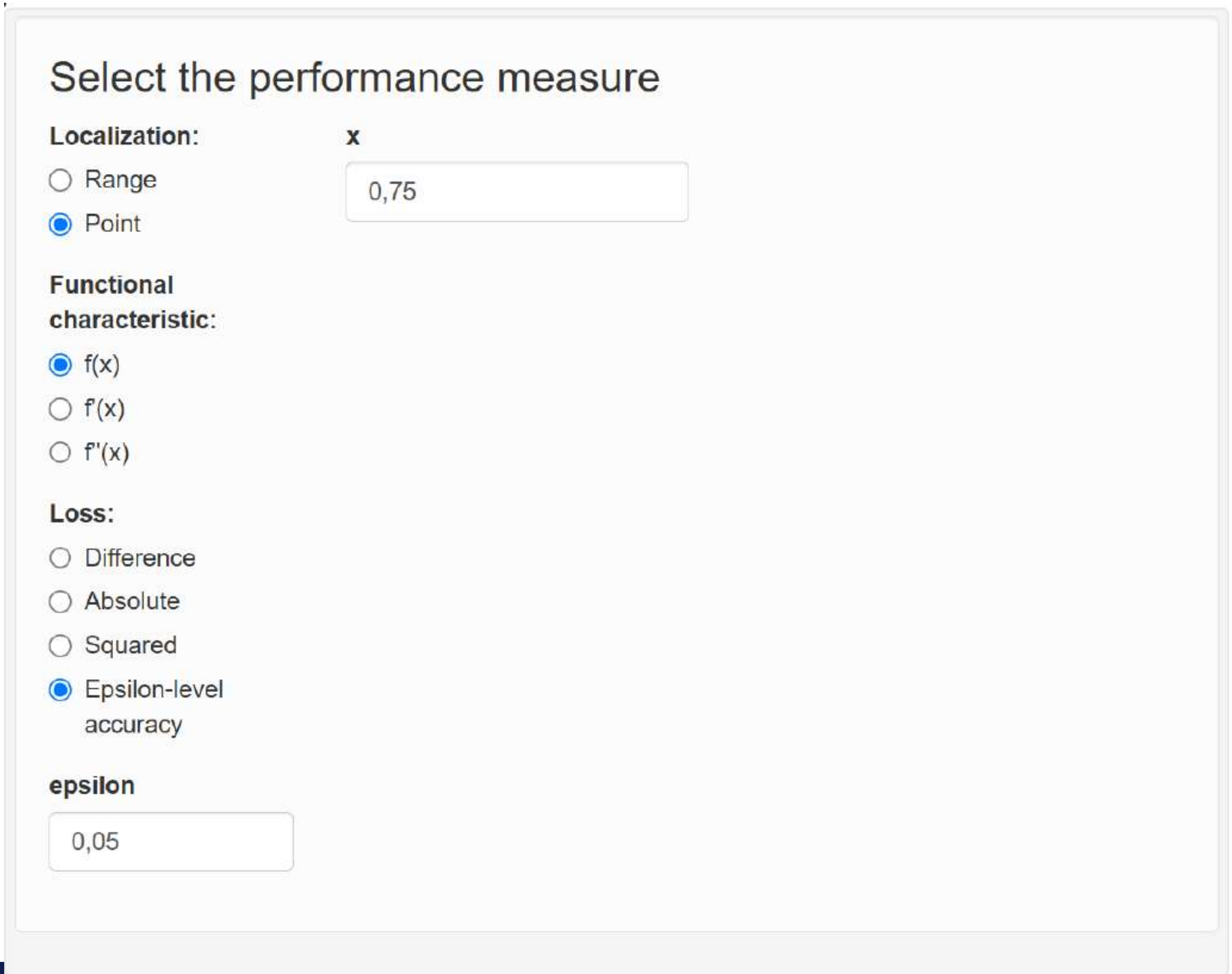
Scope of aggregration:

- (●) whole range $[0, 1]$
- ( ) subrange $[F_X^{-1}(0.05), F_X^{-1}(0.95)]$

$$= \int \left(\hat{f}(x) - f(x)\right)^2 dF(x)$$

„expected (over $F(x)$) squared deviation“

Combining these aspects



$$= I\left(\left|\hat{f}(0.75) - f(0.75)\right| \leq 0.05\right)$$

„within $f(x) \pm 0.05$ at $x = 0.75$“

Combining these aspects

Select the performance measure

Localization:

- (●) Range
- ( ) Point

Functional characteristic:

- ( ) f(x)
- ( ) f'(x)
- (●) f''(x)

Loss:

- ( ) Difference
- ( ) Absolute
- (●) Squared
- ( ) Epsilon-level accuracy

Axis of aggregation:

- (●) Y
- ( ) X

Type of aggregation:

- (●) Integration over dx
- ( ) Expectation over dF_X
- ( ) Quantile with respect to F_X
- ( ) Maximum
- ( ) Minimum

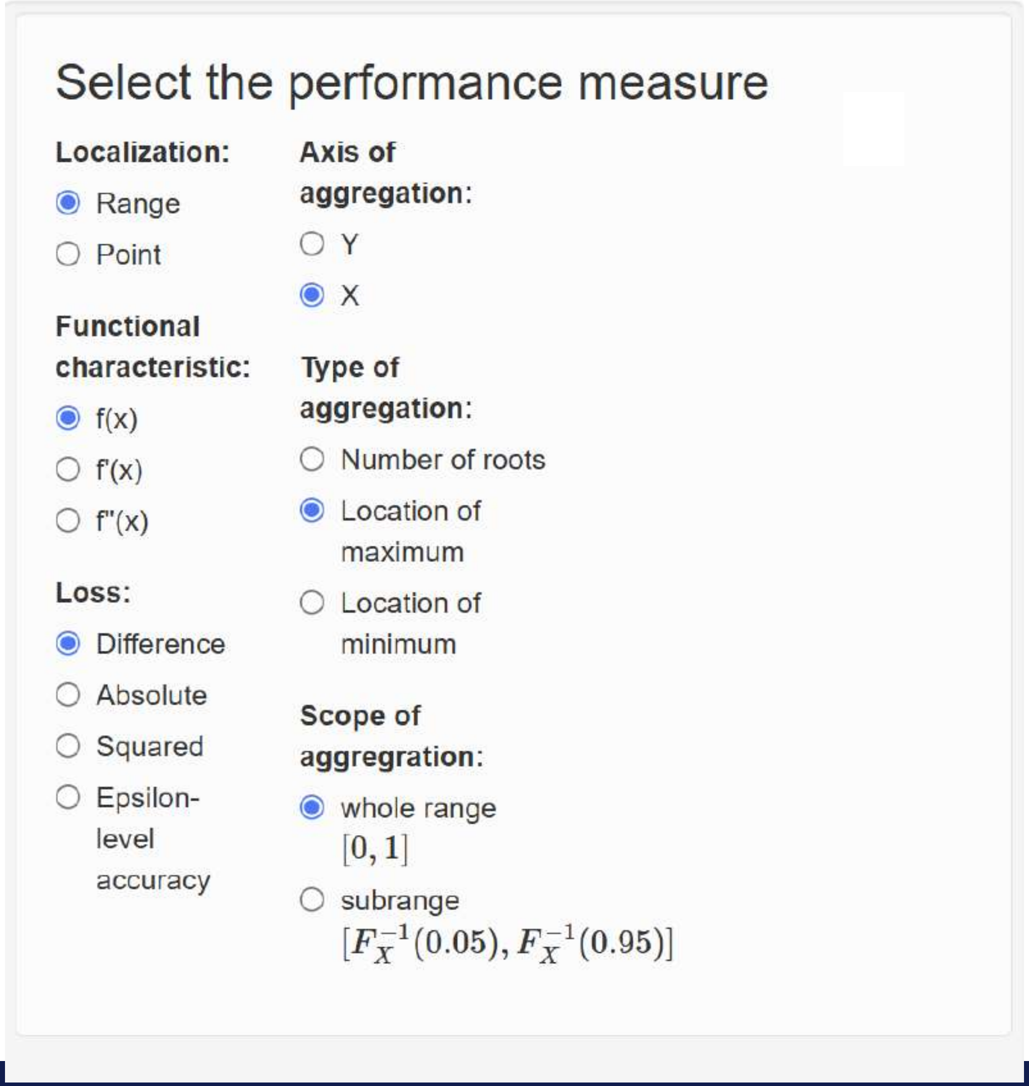
Scope of aggregration:

- ( ) whole range $[0, 1]$
- (●) subrange $[F_X^{-1}(0.05), F_X^{-1}(0.95)]$

$$=\int_{Q05}^{Q95}\left(\hat{f}''(x)-f''(x)\right)^2 dx$$

„wiggliness“

Combining these aspects



Select the performance measure

Localization:
- (●) Range
- ( ) Point

Functional characteristic:
- (●) f(x)
- ( ) f'(x)
- ( ) f"(x)

Loss:
- (●) Difference
- ( ) Absolute
- ( ) Squared
- ( ) Epsilon-level accuracy

Axis of aggregation:
- ( ) Y
- (●) X

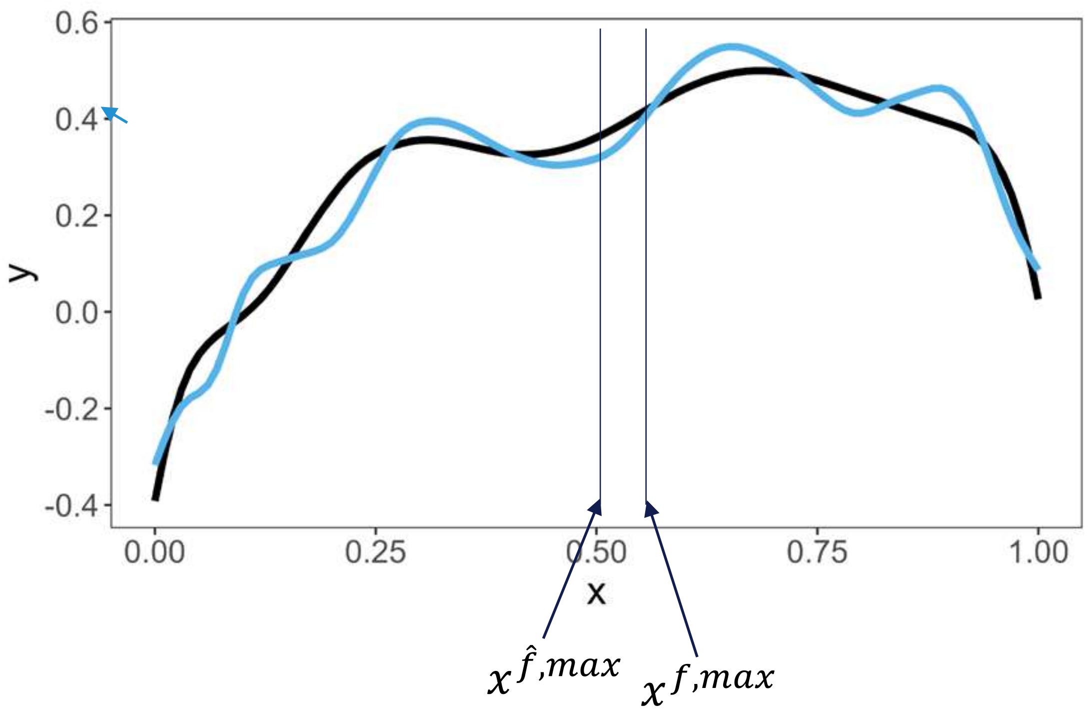
Type of aggregation:
- ( ) Number of roots
- (●) Location of maximum
- ( ) Location of minimum

Scope of aggregration:
- (●) whole range $[0, 1]$
- ( ) subrange $[F_X^{-1}(0.05), F_X^{-1}(0.95)]$

„Deviation of location of maximum“:

$$x^{\hat{f},max} - x^{f,max}$$

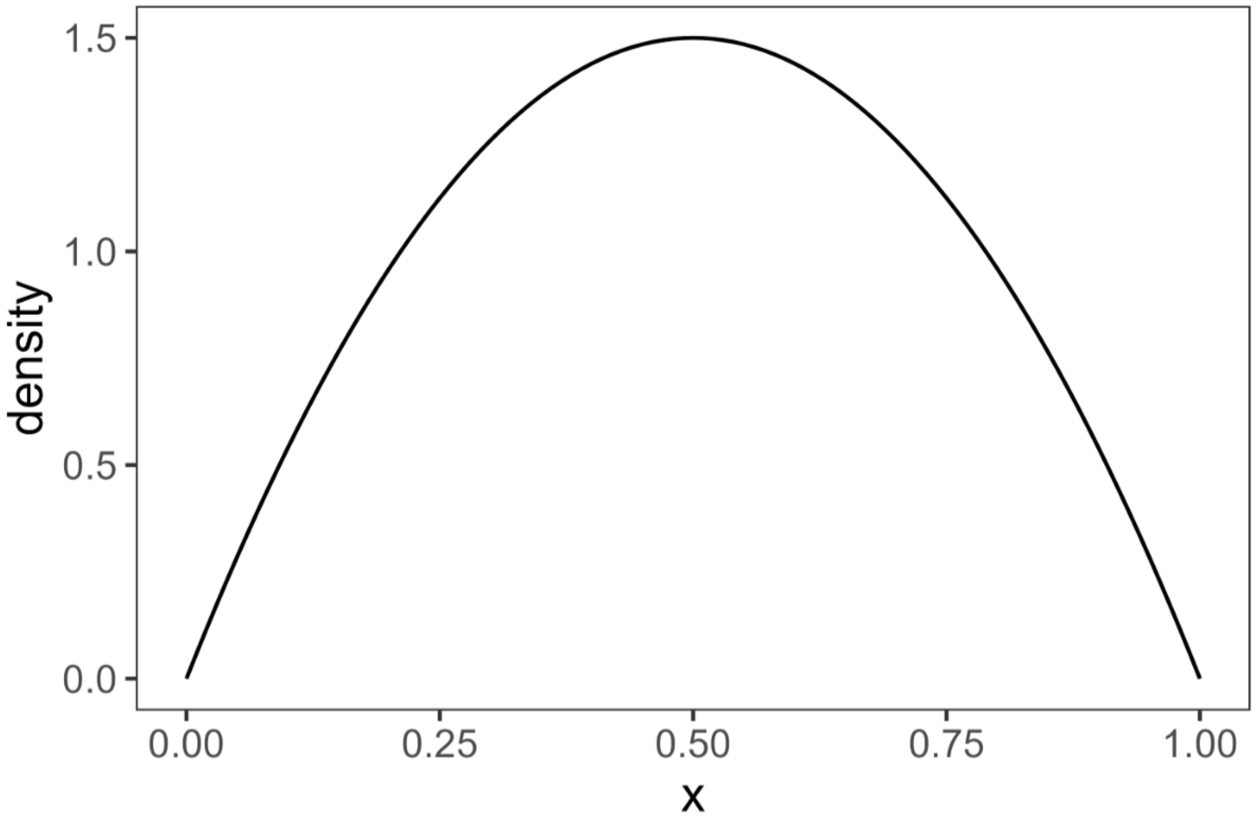
true curve f estimate $\hat{f}$

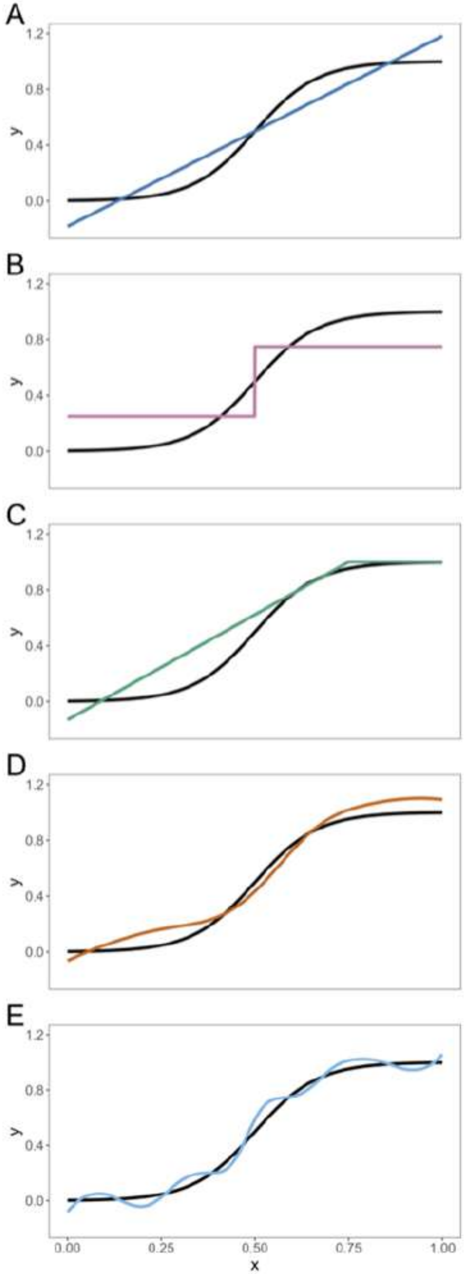


Some examples

- In these examples, we consider x distributed as Beta(2,2)

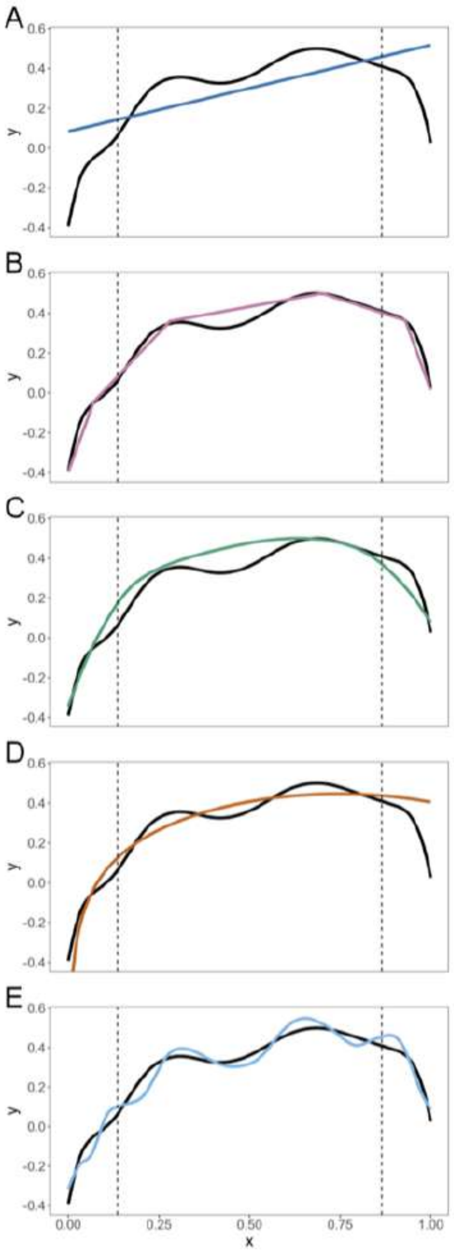
- In some examples, we will nevertheless perform the integration over dx
- In others
  we will integrate over
  dF(x)



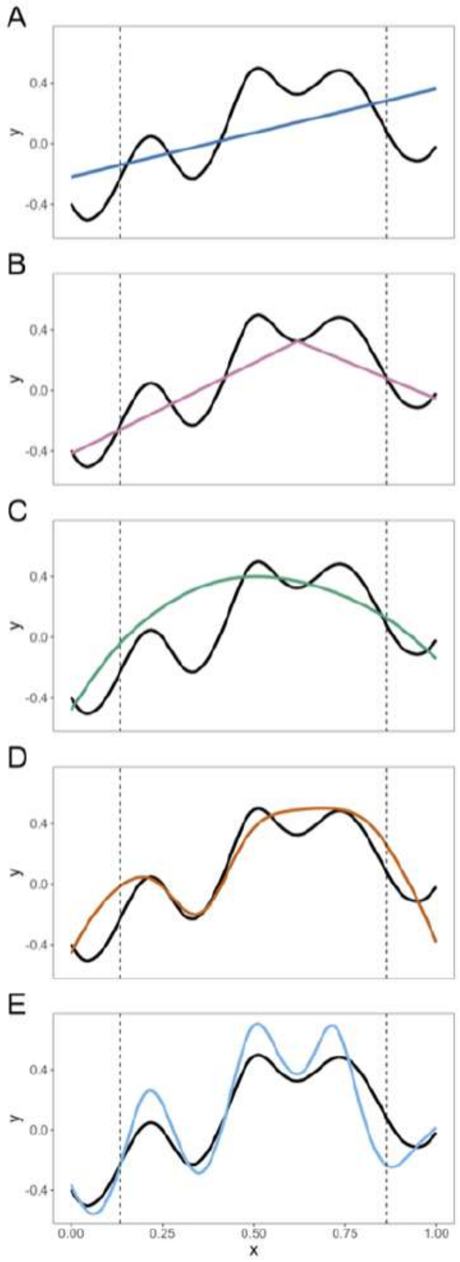


Estimate	Rank according to performance measure...		
	$\int_{\mathcal{X}} \lvert\hat{f}(x) - f(x)\rvert \mathrm{d}x$	$\int_{\mathcal{X}} \lvert\hat{f}'(x) - f'(x)\rvert \mathrm{d}x$	$\int_{\mathcal{X}} \lvert\hat{f}''(x) - f''(x)\rvert \mathrm{d}x$
A	4 (0.10)	3 (0.99)	3 (5.94)
B	5 (0.18)	4 (0.10)	3 (5.94)
C	3 (0.09)	2 (0.75)	3 (5.94)
D	2 (0.07)	1 (0.56)	1 (5.31)
E	1 (0.04)	5 (1.15)	5 (31.64)

Estimate	Rank according to performance measure... $\int_{\mathcal{X}} \lvert\hat{f}''(x) - f''(x)\rvert \mathrm{d}x$	$\int_{F_X^{-1}(0.05)}^{F_X^{-1}(0.95)} \lvert\hat{f}''(x) - f''(x)\rvert \mathrm{d}x$
A	2.5 (25.08)	2.5 (6.98)
B	2.5 (25.08)	2.5 (6.98)
C	1 (23.87)	4 (7.28)
D	5 (∞)	1 (6.58)
E	4 (43.57)	5 (15.36)

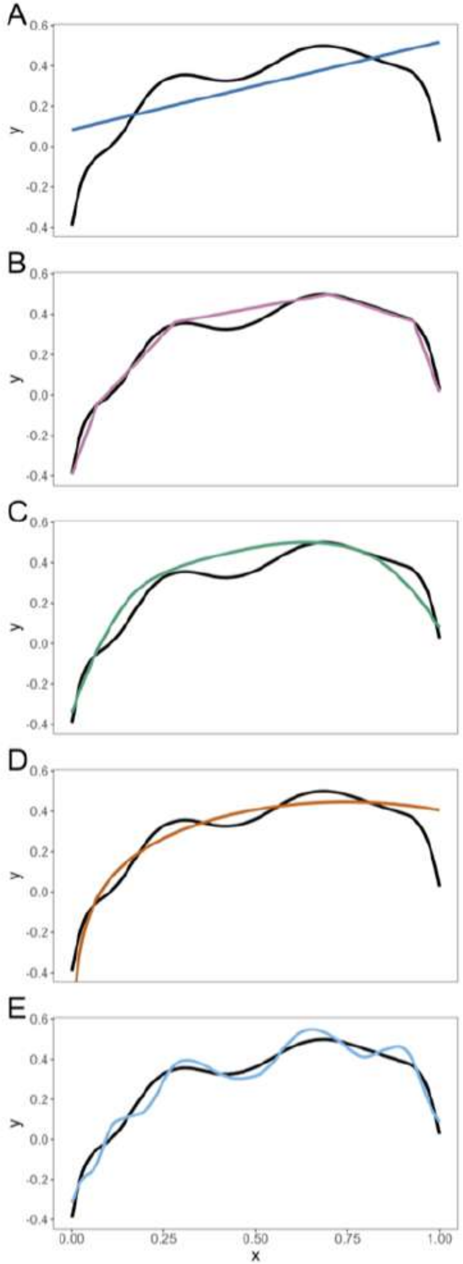


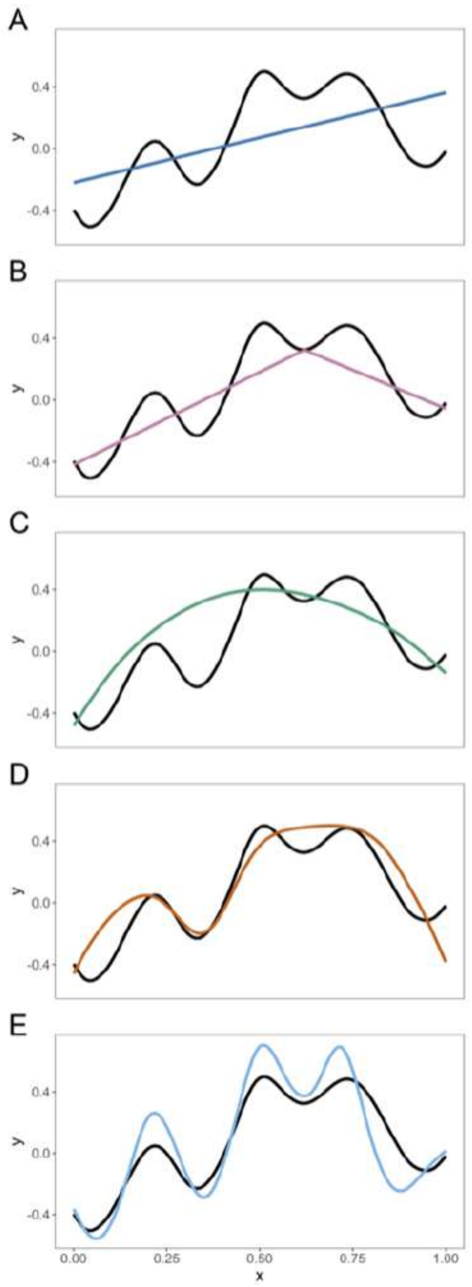
Rank according to performance measure...



Estimate	$\max_{x \in \mathcal{X}} \lvert \hat{f}(x) - f(x) \rvert$	$\max_{x \in [F_X^{-1}(0.05), F_X^{-1}(0.95)]} \lvert \hat{f}(x) - f(x) \rvert$
A	4 (0.45)	4 (0.42)
B	1 (0.31)	2 (0.31)
C	5 (0.54)	5 (0.54)
D	3 (0.37)	1 (0.21)
E	2 (0.35)	3 (0.35)

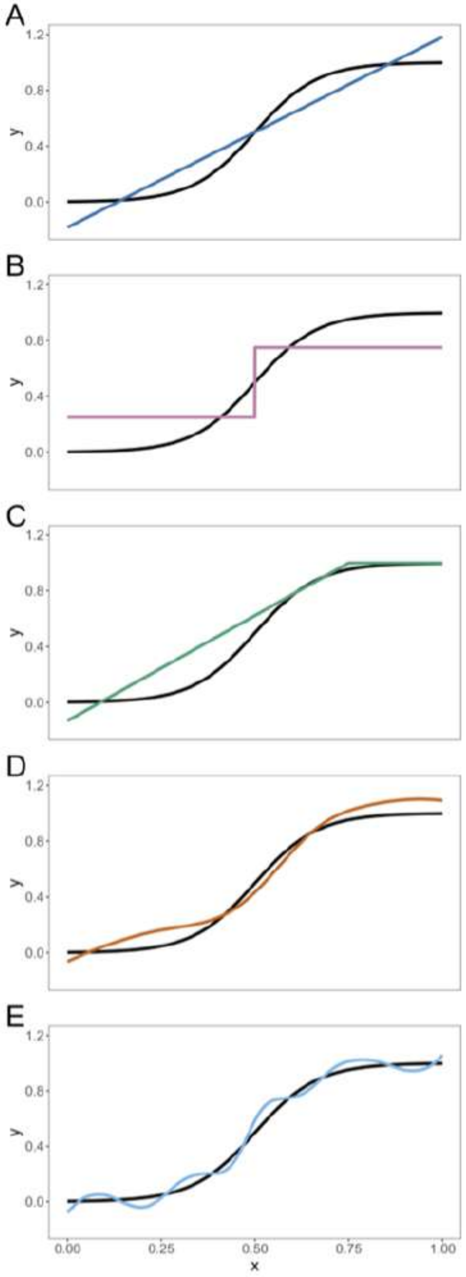
Estimate	Rank according to performance measure...	
	$\int_{\mathcal{X}} \left(\hat{f}(x) - f(x)\right)^2 \mathrm{d}x$	$\int_{\mathcal{X}} \left(\hat{f}(x) - f(x)\right)^2 \mathrm{d}F_X(x)$
A	5 (0.019)	5 (0.010)
B	1 (0.002)	2 (0.002)
C	3 (0.005)	4 (0.005)
D	4 (0.006)	3 (0.002)
E	2 (0.002)	1 (0.002)



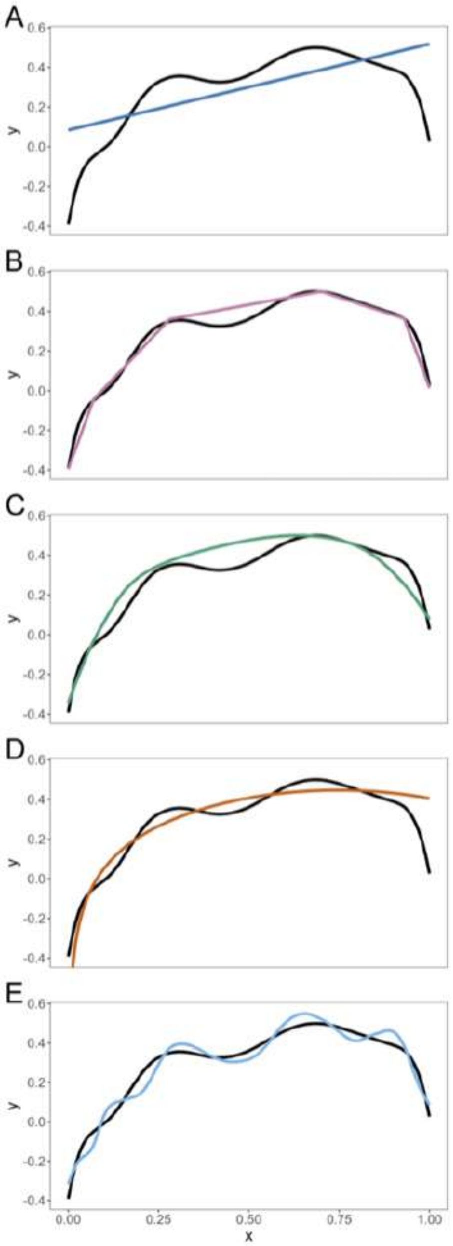


Estimate	Rank according to performance measure... $\int_{\mathcal{X}} \lvert\hat{f}(x) - f(x)\rvert \mathrm{d}x$	$\max_{x\in\mathcal{X}} \lvert\hat{f}(x) - f(x)\rvert$
A	5 (0.22)	4 (0.45)
B	3 (0.14)	1 (0.31)
C	4 (0.16)	5 (0.54)
D	1 (0.10)	3 (0.37)
E	2 (0.12)	2 (0.35)

Estimate	Rank according to performance measure...	
	$\int_{\mathcal{X}} \hat{f}(x) - f(x)\mathrm{d}x$	$\int_{\mathcal{X}} \lvert\hat{f}(x) - f(x)\rvert\mathrm{d}x$
A	1.5 (0)	4 (0.10)
B	1.5 (0)	5 (0.18)
C	5 (0.08)	3 (0.09)
D	4 (0.04)	2 (0.07)
E	3 (0.01)	1 (0.04)



Estimate	Rank according to performance measure...	
	$\int_{\mathcal{X}} \lvert \hat{f}'(x) - f'(x) \rvert \mathrm{d}F_X(x)$	$\int_{\mathcal{X}} \left(\hat{f}'(x) - f'(x) \right)^2 \mathrm{d}F_X(x)$
A	4 (0.81)	3 (1.24)
B	1 (0.46)	1 (0.42)
C	2 (0.59)	2 (0.55)
D	3 (0.66)	5 (∞)
E	5 (0.91)	4 (1.37)



How many measures are there?

According to our categorization, there are…

m = 228
performance measures

If we consider one option for the type of aggregation = "quantile with respect to F_X" (e.g., the median) and two options for the scope of aggregation ($\mathcal{X}$ and 5%-95% quantile of F_X)

m = 216
measures with
localization = "range"

m = 12
measures with
localization = "point"

→How to choose a **smaller set** of performance measures for a simulation study?

→ Select those that capture different features (see examples!)

Aggregation over simulated data sets

- Our performance measures will summarize the quality of the fitted line in 1 simulated data set
- The analyst still has to decide whether
  - Expected value of the performance measure
  - Variance of the performance measure
  - or other population quantity is of interest (e.g., median, p^{th} quantile etc.)
- If there is a clear optimum value (e.g. expected difference [=bias] should be 0), one could also construct a combination of bias + variance
  - Obvious: MSE = $bias^2$ + variance

Applications

- Univariate models: unadjusted association
- Models where the association of interest is adjusted for a (fixed) set of adjustment variables (descriptive-associational)
- Evaluation over a two-dimensional grid on X_1, X_2
- Prediction/calibration:
    - agreement of predicted and observed values
    - agreement of predicted and true linear predictor values
- Extensions: comparison to ‚null' instead of true $f(x)$
    - Number of roots
    - General wiggliness

Preprint is available on Arxiv

Ullmann, T., Heinze, G., Abrahamowicz, M., Perperoglou, A., Sauerbrei, W., Schmid, M., Dunkler, D., for TG2 of the Stratos initiative. (2025). A categorization of performance measures for estimated non-linear associations between an outcome and continuous predictors (Version 1). arXiv. https://doi.org/10.48550/ARXIV.2503.16981

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