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# Regulatory Considerations for the Treatment of Lipodystrophy – Roundtable Discussion - Forum for Collaborative HIV Research

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## Discussion areas

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- Issues in design of clinical trials for treatment of HIV-associated lipodystrophy with regard to registration of a product for approval
  - Entry criteria
  - Monitoring issues
  - Response criteria

# Conditions reviewed

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- Facial lipoatrophy
- Peripheral lipoatrophy
- Visceral fat gain
- Dorsocervical fat pads

# Facial lipoatrophy

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- Entry criteria
  - Patient and physician defined
- Baseline measurement
  - Objective - ultrasound, photography
- Response criteria
  - Overall appearance improved
  - Objective change – standardized measure/grading scale
  - Subjective improvement
    - QOL, Visual score
    - Needs to be blinded if possible
- Durability critical
  - At least 2 years of follow-up

# Peripheral lipoatrophy

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- Entry criteria
  - Subjective entry – patient and doctor agree
- Baseline measurement
  - Objective - DXA, CT
- Response
  - % change from baseline
  - If severe LA, small amount increase enough (10-15%), especially if also metabolic improvement
  - Moderate LA, need substantial improvement (e.g. 40% over 2 yrs), but that alone may be sufficient
  - Subjective assessment if improvement slow may be problem
  - Does not worsen fat gain

# Visceral fat Gain

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- Entry criteria
  - Anthropometry, WC and WHR
    - Adjusted by gender
- Baseline measurement
  - objective - CT +/- DEXA
  - QOL
- Response
  - % change from baseline – 8-10% decrease in VAT
  - Lipoatrophy does not worsen
  - Metabolic changes at least neutral
  - Subjective assessment (blinded)

# Dorsocervical fat pad

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- Entry criteria
  - Subjective entry – patient and doctor agree
- Baseline measurement
  - Anthropomorphic measurement
  - Symptom score (specific)
- Response
  - % change from baseline
  - Subjective assessment , QOL, symptom score

# All studies

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- Safety issues
  - Long-term and short-term
- Impact on HIV and ART
  - No effect on VL or CD4
  - Drug interaction data on commonly used ART



# Participants

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Herbert Lerner

Jeff Murray

Robert Perlstein

Stephen Rhodes

Kimberly Struble

Michael de Baar

Alex DePaoli

Ronald Gottschalk

Robert Kirsch

Sharon Levy

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Denise Sutherland -Phillips

Jacqueline Capeau

Andrew Carr

Ben Cheng

Simon Collins

David Cooper

Carl Fichtenbaum

Carl Grunfeld

Esteban Martinez

Veronica Miller

Kathy Mulligan

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